

APPLIED GENETIC TECHNOLOGIES CORP  
Form 10-Q  
February 07, 2019

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**  
**WASHINGTON, DC 20549**

**FORM 10-Q**

(Mark One)

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

**For the quarterly period ended December 31, 2018**

**OR**

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

**Commission File Number: 001-36370**

**APPLIED GENETIC**  
**TECHNOLOGIES CORPORATION**  
**(Exact Name of Registrant as Specified in Its Charter)**

**Delaware**  
**(State or Other Jurisdiction of**  
**Incorporation or Organization)**  
**14193 NW 119<sup>th</sup> Terrace**  
**Suite 10**  
**Alachua, Florida 32615**  
**(Address of Principal Executive Offices, Including Zip Code)**  
**(386) 462-2204**  
**(Registrant's Telephone Number, Including Area Code)**

**59-3553710**  
**(I.R.S. Employer**  
**Identification No.)**

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 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

Accelerated filer

Non-accelerated filer

Smaller reporting company

Emerging growth company

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

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

The number of shares of the registrant's common stock outstanding as of February 7, 2019 was 18,165,054.



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**APPLIED GENETIC TECHNOLOGIES CORPORATION**

**FORM 10-Q**

**FOR THE QUARTER ENDED DECEMBER 31, 2018**

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**SIGNATURES**

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**PART I. FINANCIAL INFORMATION**
**ITEM 1. FINANCIAL STATEMENTS****APPLIED GENETIC TECHNOLOGIES CORPORATION****CONDENSED BALANCE SHEETS****(Unaudited)**

| In thousands, except per share data  | <b>December 31,<br/>2018</b> | <b>June 30,<br/>2018</b> |
|--|------------------------------|--------------------------|
| <b>ASSETS</b>  |                              |                          |
| <b>Current assets:</b>   |                              |                          |
| Cash and cash equivalents  | \$ 23,978                    | \$ 31,065                |
| Investments  | 72,097                       | 73,840                   |
| Grants receivable  | 116                          | 210                      |
| Prepaid and other current assets   | 2,959                        | 4,009                    |
| <b>Total current assets</b>  | <b>99,150</b>                | <b>109,124</b>           |
| Property and equipment, net  | 4,778                        | 5,254                    |
| Intangible assets, net   | 954                          | 968                      |
| Investment in Bionic Sight   | 1,961                        | 1,980                    |
| Other assets   | 1,260                        | 1,206                    |
| <b>Total assets</b>  | <b>\$ 108,103</b>            | <b>\$ 118,532</b>        |
| <b>LIABILITIES AND STOCKHOLDERS EQUITY</b>   |                              |                          |
| <b>Current liabilities:</b>  |                              |                          |
| Accounts payable   | \$ 1,664                     | \$ 945                   |
| Accrued and other liabilities  | 5,823                        | 7,155                    |
| Deferred revenue   | 12,701                       | 6,295                    |
| <b>Total current liabilities</b>   | <b>20,188</b>                | <b>14,395</b>            |
| Deferred revenue, net of current portion   | 7,731                        | 610                      |
| Other liabilities  | 4,209                        | 4,345                    |
| <b>Total liabilities</b>   | <b>32,128</b>                | <b>19,350</b>            |
| <b>Stockholders equity:</b>  |                              |                          |
| Preferred stock, par value \$.001 per share, 5,000 shares authorized, no shares issued and outstanding   |                              |                          |
| Common stock par value \$.001 per share; 150,000 shares authorized; 18,179 and 18,137 share issued; 18,164 and 18,126 shares outstanding at December 31, and June 30, 2018, respectively | 18                           | 18                       |

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|   |                   |                   |
|---|-------------------|-------------------|
| Additional paid-in capital  | 212,550           | 210,139           |
| Shares held in treasury of 15 and 11 at December 31, 2018 and June 30, 2018, respectively | (70)              | (49)              |
| Accumulated deficit   | (136,523)         | (110,926)         |
| Total stockholders' equity  | 75,975            | 99,182            |
| <b>Total liabilities and stockholders' equity</b>   | <b>\$ 108,103</b> | <b>\$ 118,532</b> |

The accompanying notes are an integral part of these Unaudited Condensed Financial Statements.

**APPLIED GENETIC TECHNOLOGIES CORPORATION**

**CONDENSED STATEMENTS OF OPERATIONS**

(Unaudited)

| In thousands, except per share amounts        | For the Three Months<br>Ended December 31, |            | For the Six Months<br>Ended<br>December 31, |            |
|---|--|------------|---|------------|
|   | 2018                                       | 2017       | 2018  | 2017       |
| <b>Revenue:</b>                               |  |            |   |            |
| Collaboration revenue                         | \$ 5,895                                   | \$ 4,831   | \$ 19,920                                   | \$ 15,139  |
| Grant and other revenue                       | 39   | 21         | 48  | 28         |
| Total revenue                                 | 5,934                                      | 4,852      | 19,968                                      | 15,167     |
| <b>Operating expenses:</b>                    |  |            |   |            |
| Research and development                      | 7,583                                      | 7,726      | 17,648                                      | 16,002     |
| General and administrative and other          | 3,022                                      | 3,368      | 6,235                                       | 7,074      |
| Total operating expenses                      | 10,605                                     | 11,094     | 23,883                                      | 23,076     |
| Loss from operations                          | (4,671)                                    | (6,242)    | (3,915)                                     | (7,909)    |
| <b>Other income:</b>                          |  |            |   |            |
| Investment income, net                        | 520  | 271        | 991   | 541        |
| Other expense                                 |  | (10)       |   | (10)       |
| Total other income, net                       | 520  | 261        | 991   | 531        |
| Loss before provision for income taxes        | (4,151)                                    | (5,981)    | (2,924)                                     | (7,378)    |
| Provision (benefit) for income taxes          | 19   | (791)      | 38  | (791)      |
| Loss before equity in net losses of affiliate | (4,170)                                    | (5,190)    | (2,962)                                     | (6,587)    |
| Equity in net losses of affiliate             | (11)                                       |            | (19)  |            |
| Net loss                                      | \$ (4,181)                                 | \$ (5,190) | \$ (2,981)                                  | \$ (6,587) |
| <b>Weighted Average Shares Outstanding</b>    |  |            |   |            |
| Weighted average shares outstanding basic     | 18,151                                     | 18,094     | 18,140                                      | 18,091     |
| Weighted average shares outstanding diluted   | 18,151                                     | 18,094     | 18,140                                      | 18,091     |
| <b>Net loss per common share</b>              |  |            |   |            |
| Net loss per share, basic                     | \$ (0.23)                                  | \$ (0.29)  | \$ (0.16)                                   | \$ (0.36)  |
| Net loss per share, diluted                   | \$ (0.23)                                  | \$ (0.29)  | \$ (0.16)                                   | \$ (0.36)  |

The accompanying notes are an integral part of these Unaudited Condensed Financial Statements.





**APPLIED GENETIC TECHNOLOGIES CORPORATION**  
**CONDENSED STATEMENT OF STOCKHOLDERS EQUITY**

(Unaudited)

| In thousands  | Common Stock       |        | Treasury Stock     |         | Additional Paid-in Capital | Accumulated Deficit | Total      |
|---|--------------------|--------|--------------------|---------|----------------------------|---------------------|------------|
|   | Outstanding Shares | Amount | Outstanding Shares | Amount  |                            |                     |            |
| <b>Balance, June 30, 2017</b>                           | 18,088             | \$ 18  |                    | \$      | \$ 204,937                 | \$ (89,626)         | \$ 115,329 |
| Share based compensation expense                        |                    |        |                    |         | 1,469                      |                     | 1,469      |
| Shares issued under employee plans                      | 5                  |        |                    |         | 2                          |                     | 2          |
| Net loss  |                    |        |                    |         |                            | (1,397)             | (1,397)    |
| <b>Balance, September 30, 2017</b>                      | 18,093             | \$ 18  |                    | \$      | \$ 206,408                 | \$ (91,023)         | \$ 115,403 |
| Share based compensation expense                        |                    |        |                    |         | 1,331                      |                     | 1,331      |
| Shares issued under employee plans                      | 12                 |        |                    |         | 39                         |                     | 39         |
| Net loss  |                    |        |                    |         |                            | (5,190)             | (5,190)    |
| <b>Balance, December 31, 2017</b>                       | 18,105             | \$ 18  |                    | \$      | \$ 207,778                 | \$ (96,213)         | \$ 111,583 |
| <b>Balance, June 30, 2018</b>                           | 18,126             | \$ 18  | 11                 | \$ (49) | \$ 210,139                 | \$ (110,926)        | \$ 99,182  |
| Cumulative impact of adopting Topic 606 on July 1, 2018 |                    |        |                    |         |                            | (22,616)            | (22,616)   |
| Share based compensation expense                        |                    |        |                    |         | 1,181                      |                     | 1,181      |
| Shares issued under employee plans                      | 4                  |        | 2                  | (8)     |                            |                     | (8)        |
| Net income  |                    |        |                    |         |                            | 1,200               | 1,200      |
| <b>Balance, September 30, 2018</b>                      | 18,130             | \$ 18  | 13                 | \$ (57) | \$ 211,320                 | \$ (132,342)        | \$ 78,939  |
| Share based compensation expense                        |                    |        |                    |         | 1,094                      |                     | 1,094      |
| Shares issued under employee plans                      | 34                 |        | 2                  | (13)    | 136                        |                     | 123        |
| Net loss  |                    |        |                    |         |                            | (4,181)             | (4,181)    |
| <b>Balance, December 31, 2018</b>                       | 18,164             | \$ 18  | 15                 | \$ (70) | \$ 212,550                 | \$ (136,523)        | \$ 75,975  |

The accompanying notes are an integral part of these Unaudited Condensed Financial Statements.



**APPLIED GENETIC TECHNOLOGIES CORPORATION**

**CONDENSED STATEMENTS OF CASH FLOWS**

(Unaudited)

| In thousands  | <b>For the Six Months<br/>Ended December 31,</b> |                  |
|---|--|------------------|
|   | <b>2018</b>                                      | <b>2017</b>      |
| <b>Cash flows from operating activities</b>                                   |  |                  |
| Net loss  | \$ (2,981)                                       | \$ (6,587)       |
| Adjustments to reconcile net loss to net cash (used in) operating activities: |  |                  |
| Share-based compensation expense  | 2,275  | 2,800            |
| Depreciation and amortization   | 639  | 544              |
| Recovery of bad debts   | (258)  |                  |
| Investment (discount accretion) premium amortization                          | (353)  | 143              |
| Equity in net losses of affiliate   | 19   |                  |
| Changes in operating assets and liabilities:                                  |  |                  |
| Grants receivable   | (17)   | (29)             |
| Prepaid and other assets  | 291  | (1,747)          |
| Deferred revenues   | (7,960)  | (13,489)         |
| Accounts payable  | 719  | 38               |
| Accrued and other liabilities   | (1,350)  | (173)            |
| <b>Net cash (used in) operating activities</b>                                | <b>(8,976)</b>                                   | <b>(18,500)</b>  |
| <b>Cash flows from investing activities</b>                                   |  |                  |
| Purchase of property and equipment  | (81)   | (134)            |
| Purchase of and capitalized costs related to intangible assets                | (68)   |                  |
| Maturity of investments   | 46,619   | 58,288           |
| Purchase of investments   | (44,523)   |                  |
| <b>Net cash provided by investing activities</b>                              | <b>1,947</b>                                     | <b>58,154</b>    |
| <b>Cash flows from financing activities</b>                                   |  |                  |
| Proceeds from exercise of common stock options                                | 136  | 3                |
| Deferred offering costs   | (168)  |                  |
| Payments made toward capital lease obligations                                | (26)   | (18)             |
| <b>Net cash (used in) financing activities</b>                                | <b>(58)</b>                                      | <b>(15)</b>      |
| <b>Net change in cash and cash equivalents</b>                                | <b>(7,087)</b>                                   | <b>39,639</b>    |
| <b>Cash and cash equivalents, beginning of period</b>                         | <b>31,065</b>                                    | <b>30,706</b>    |
| <b>Cash and cash equivalents, end of period</b>                               | <b>\$ 23,978</b>                                 | <b>\$ 70,345</b> |

**Supplemental disclosure of cash flow**

|  |    |    |       |
|--|----|----|-------|
| Cash paid during the period for income taxes                                 | \$ | \$ | 670   |
| <b>Non cash flow information</b>   |    |    |       |
| Capital lease obligation related to the purchase of equipment                | \$ | \$ | 209   |
| Lease incentive obligation related to the purchase of leasehold improvements | \$ | \$ | 627   |
| Shares issued for no consideration   | \$ | 21 | \$ 38 |

The accompanying notes are an integral part of these Unaudited Condensed Financial Statements.

**APPLIED GENETIC TECHNOLOGIES CORPORATION**

**NOTES TO CONDENSED FINANCIAL STATEMENTS (UNAUDITED)**

**1. Organization and Operations:**

Applied Genetic Technologies Corporation (the Company or AGTC) was incorporated as a Florida corporation on January 19, 1999 and reincorporated as a Delaware corporation on October 24, 2003. The Company is a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases.

In July 2015, the Company entered into a collaboration agreement (the Collaboration Agreement) with Biogen MA, Inc., a wholly owned subsidiary of Biogen Inc. (Biogen), pursuant to which the Company and Biogen will collaborate to develop, seek regulatory approval for and commercialize gene therapy products to treat X-linked retinoschisis (XLRS), X-linked retinitis pigmentosa (XLRP), and discovery programs targeting three indications based on the Company's adeno-associated virus vector technologies. The Collaboration Agreement became effective in August 2015. On December 7, 2018, the Company received notice from Biogen that it had elected to terminate the Collaboration Agreement effective as of March 8, 2019. The Collaboration Agreement and other transactions with Biogen are discussed further in Note 6 to these Unaudited Condensed Financial Statements.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not completed the development of any products. The Company has generated revenue from collaboration agreements, sponsored research payments and grants, but has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies in the biotechnology industry, including dependence on key individuals, the difficulties inherent in the development of commercially viable products, the need to obtain additional capital necessary to fund the development of its products, development by the Company or its competitors of technological innovations, risks of failure of clinical studies, protection of proprietary technology, compliance with government regulations and ability to transition to large-scale production of products. As of December 31, 2018, the Company had an accumulated deficit of \$136.5 million. While the Company expects to continue to generate some revenue from partnering, the Company expects to incur losses for the foreseeable future. The Company has funded its operations to date primarily through public offerings of its common stock, private placements of its preferred stock, and collaborations. At December 31, 2018, the Company had cash and cash equivalents and liquid investments of \$96.1 million.

## **2. Summary of Significant Accounting Policies:**

### ***Basis of presentation***

The accompanying Unaudited Condensed Financial Statements have been prepared in conformity with accounting principles generally accepted in the United States of America ( GAAP ) and, in the opinion of management, include all adjustments necessary for a fair presentation of the Company's financial position, results of operations, stockholders equity and cash flows for the periods presented.

The adjustments referred to above are of a normal and recurring nature. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to U.S. Securities and Exchange Commission ( SEC ) rules and regulations for interim reporting.

The Condensed Balance Sheet as of June 30, 2018 was derived from audited financial statements, but does not include all disclosures required by GAAP. These Unaudited Condensed Financial Statements should be read in conjunction with the audited financial statements included in the Company's 2018 Annual Report on Form 10-K ( June 30, 2018 Form 10-K ). Results of operations for the three and six months ended December 31, 2018 are not necessarily indicative of the results to be expected for the full year or any other interim period.

### ***Segment reporting***

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker in making decisions regarding resource allocation and assessing performance. To date, we have viewed our operations and managed our business as one segment.

### ***Use of estimates***

The preparation of financial statements in conformity with GAAP and SEC regulations, requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amount of revenues and expenses during the reporting period. Actual results could differ from those estimates.

### ***Cash and cash equivalents***

Cash consists of funds held in bank accounts. Cash equivalents consist of short-term, highly liquid investments with original maturities of 90 days or less at the time of purchase and generally include money market accounts.

### ***Investments***

The Company's investments consist of certificates of deposit and debt securities classified as held-to-maturity. Management determines the appropriate classification of debt securities at the time of purchase and reevaluates such designation as of each balance sheet date. Debt securities are classified as held-to-maturity when the Company has the positive intent and ability to hold the securities to maturity. Held-to-maturity securities are stated at amortized cost, adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion is included in investment income. Interest on securities classified as held-to-maturity is included in investment income.

The Company uses the specific identification method to determine the cost basis of securities sold.

Investments are considered to be impaired when a decline in fair value is judged to be other-than-temporary. The Company evaluates an investment for impairment by considering the length of time and extent to which market value has been less than cost or amortized cost, the financial condition and near-term prospects of the issuer as well as specific events or circumstances that may influence the operations of the issuer and the Company's intent to sell the security or the likelihood that it will be required to sell the security before recovery of the entire amortized cost. Once a decline in fair value is determined to be other-than-temporary, an impairment charge is recorded to investment income (expense) and a new cost basis in the investment is established.

### ***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. The Financial Accounting Standards Board (FASB) Accounting Standard Codification (ASC) Topic 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs 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 financial instruments 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.

### ***Revenue recognition***

Effective July 1, 2018, the Company adopted the provisions of ASC Topic 606, *Revenue from Contracts with Customers*, (Topic 606), using the modified retrospective transition method. Under this method, the Company

recorded the cumulative effect of initially applying the new standard to all contracts in process as of the date of adoption. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards.



The adoption of the new revenue recognition guidance resulted in an increase of \$22.6 million in deferred revenue and accumulated deficit as of July 1, 2018. For the six months ended December 31, 2018, revenue increased by \$5.0 million, net income increased by \$5.0 million and basic and diluted net income per share increased by \$0.28 per share based on revenue recognition under Topic 606 as compared to the Company's prior revenue recognition methodology under ASC 605, *Revenue Recognition*. These changes were primarily caused by the differences in determining and allocating transaction price and recognizing revenue on a proportional performance basis under Topic 606.

The Company may enter into collaboration agreements which are within the scope of Topic 606, under which the Company licenses rights to its technology and certain of the Company's product candidates and performs research and development services for third parties. The terms of these arrangements typically may include payment of one or more of the following: non-refundable, up-front fees; reimbursement of research and development costs; development, regulatory and commercial milestone payments; and royalties on net sales of licensed products.

Under Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of Topic 606, the Company performs the following five steps: (i) identification of the contract; (ii) determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect consideration it is entitled to in exchange for the goods or services it transfers to the customer.

Performance obligations are promises to transfer distinct goods or services to the customer. Promised goods or services are considered distinct when (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on their own or whether the required expertise is readily available.

The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include both fixed consideration or variable consideration. At the inception of an arrangement that includes variable consideration and at each reporting period, the Company evaluates the amount of potential payment and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected amount method to estimate the amount to be received based on which method better predicts the amount expected to be received. If it is probable that a significant revenue reversal would not occur, the variable consideration is included in the transaction price. The Company will assess its revenue generating arrangements in order to determine whether a significant financing component exists and conclude that a significant financing component does not exist in any of its arrangements if: (a) the promised consideration approximates the cash selling price of the promised goods and services or any significant difference is due to factors other than financing; and (b) timing of payment approximates the transfer of goods and services and performance is over a relatively short period of time within the context of the entire term of the contract.

The Company's contracts will often include development and regulatory milestone payments. At contract inception and at each reporting period, the Company evaluates whether the milestones are considered probable of being reached 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 would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's control or the customer's control, such as regulatory approvals, are not included in the transaction price. At the end of each subsequent reporting period, the Company re-evaluates the

probability of achievement of such development milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues and earnings in the period of adjustment.

For arrangements that may include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of the Company's collaboration arrangements.

The Company allocates the transaction price based on the estimated standalone selling price of the underlying performance obligations or in the case of certain variable consideration to one or more performance obligations. The Company must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. The Company utilizes key assumptions to determine the stand-alone selling price, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs to complete the respective performance obligation. Certain variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated to each performance obligation are consistent with the amounts the Company would expect to receive for each performance obligation.

For performance obligations consisting of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, up-front fees. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. If the license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company will recognize revenue from non-refundable, up-front fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license.

The Company receives payments from its customers based on billing terms established in each contract. Such billings generally have 30-day payment terms. Upfront payments and fees are recorded as deferred revenue upon receipt or when due until the Company performs its obligations under these arrangements. Amounts are recorded as accounts receivable when the right to consideration is unconditional.

### ***Income taxes***

The Company uses the asset and liability method for accounting for income taxes. Under this method, deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective income tax bases. Deferred tax assets and liabilities are measured using enacted rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled.

The Tax Cut and Jobs Act (the "Tax Act") was enacted on December 22, 2017. The Tax Act contains several key provisions including, among other things, reducing the U.S. federal corporate tax rate from 35% to 21%. In addition, federal net operating losses ("NOLs") will be carried forward indefinitely, but will be subject to an 80% utilization against taxable income. The Company has enacted the reduction in tax rate effective January 1, 2018, which resulted in a decrease to the deferred tax asset and a decrease to the valuation allowance. During the second quarter of fiscal 2019, the measurement period provided by SEC Staff Accounting Bulletin 118 closed and the Company did not make any other adjustments to the provisional estimates recorded in prior periods. Although the measurement period has closed, further technical guidance related to the Tax Act, including final regulations on a broad range of topics, is expected to be issued. In accordance with Accounting Standards Codification (ASC) 740, the Company will recognize any effects of the guidance in the period that such guidance is issued.

As required by U.S. GAAP, the Company recognizes the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority. Any interest and penalties related to uncertain tax positions will be reflected in income tax expense. The

Company is subject to examination of its income tax returns in the federal and state income tax jurisdictions in which it operates. On December 28, 2015, the United States Internal Revenue Service, or IRS, notified the Company of an income tax audit for the tax period ending June 30, 2014. As of June 30, 2017, the IRS audit was closed and the Company incurred no penalties or payment liabilities for its income tax positions.

For the six months ended December 31, 2018, the Company's tax expense included an increase in the uncertain tax position liability of \$38,000 related to interest on the uncertain tax position. The uncertain tax position liability as of December 31, 2018 and June 30, 2018 was \$1,997,000 and \$1,959,000, respectively.

### ***Research and development***

Research and development costs include costs incurred in identifying, developing and testing product candidates and generally comprise compensation and related benefits and non-cash share-based compensation to research related employees; laboratory costs; animal and laboratory maintenance and supplies; rent; utilities; clinical and pre-clinical expenses; and payments for sponsored research, scientific and regulatory consulting fees and testing.

As part of the process of preparing its financial statements, the Company is required to estimate its accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on its behalf and estimating the level of service performed and the associated cost incurred for services for which the Company has not yet been invoiced or otherwise notified of the actual cost. The majority of the Company's service providers invoice the Company monthly in arrears for services performed or when contractual milestones are met. The Company makes estimates of its accrued expenses as of each balance sheet date in its financial statements based on facts and circumstances known to it at that time. The significant estimates in the Company's accrued research and development expenses are related to expenses incurred with respect to academic research centers, contract research organizations, and other vendors in connection with research and development activities for which it has not yet been invoiced.

There may be instances in which the Company's service providers require advance payments at the inception of a contract or in which payments made to these vendors will exceed the level of services provided, resulting in a prepayment of the research and development expense. Such prepayments are charged to research and development expense as and when the service is provided or when a specific milestone outlined in the contract is reached.

Prepayments related to research and development activities were \$1.3 million and \$1.0 million at December 31, 2018 and June 30, 2018, respectively, and are included within the prepaid and other current assets line item on the Unaudited Condensed Balance Sheets.

### ***Share-based compensation***

The Company accounts for share-based awards issued to employees in accordance with ASC Topic 718, *Compensation - Stock Compensation* and generally recognizes share-based compensation expense on a straight-line basis over the periods during which the employees are required to provide service in exchange for the award. In addition, the Company issues stock options and restricted shares of common stock to non-employees in exchange for consulting services and accounts for these in accordance with the provisions of ASC Subtopic 505-50, *Equity-Based Payments to Non-employees* (ASC 505-50). Under ASC 505-50, share-based awards to non-employees are subject to periodic fair value re-measurement over their vesting terms.

For purposes of calculating stock-based compensation, the Company estimates the fair value of stock options using a Black-Scholes option-pricing model. The determination of the fair value of share-based payment awards utilizing the Black-Scholes model is affected by the Company's stock price and a number of assumptions, including expected volatility, expected life, risk-free interest rate and expected dividends. Historically, the expected volatility was primarily based on the historical volatility of peer company data. For the three and six months ended December 31,

2018, the expected volatility is based on the historical volatility of the company stock price. If the Company had used peer company data for the three and six months ended December 31, 2018, share-based compensation expense for the reporting period would have differed by an insignificant amount. The expected life of the stock options is based on historical and other economic data trended into the future. The risk-free interest rate assumption is based on observed interest rates appropriate for the expected terms of the Company's stock options. The dividend yield

assumption is based on the Company's history and expectation of no dividend payouts. If factors change and the Company employs different assumptions, stock-based compensation expense may differ significantly from what has been recorded in the past. If there is a difference between the assumptions used in determining stock-based compensation expense and the actual factors which become known over time, specifically with respect to anticipated forfeitures, the Company may change the input factors used in determining stock-based compensation costs for future grants. These changes, if any, may materially impact the Company's results of operations in the period such changes are made.

### ***Net loss per share***

Basic net loss per share is calculated by dividing net loss by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the diluted net loss per share calculation, stock options and warrants are considered to be common stock equivalents. The dilutive impact of stock options and warrants for the three and six month periods ended December 31, 2018 and 2017 totaled 0.2 million shares. The dilutive impact of stock options and warrants have been excluded from the calculation of diluted net loss per share for the for the three and six months ended December 31, 2018 and December 31, 2017, as their effect would be anti-dilutive. Therefore, for the three and six months ended December 31, 2018 and December 31, 2017, basic and diluted net loss per share are the same.

### ***Comprehensive income or loss***

Comprehensive income (loss) consists of net income (loss) and changes in equity during a period from transactions and other equity and circumstances generated from non-owner sources. The Company's net loss equals comprehensive loss for all periods presented.

### ***New accounting pronouncements***

#### ***Adopted in the current period***

#### **Revenue recognition**

In May 2014, Topic 606, which replaces the existing accounting standards for revenue recognition with a single comprehensive five-step model. The core principle is to recognize revenue upon the transfer of goods or services to customers at an amount that reflects the consideration expected to be received. It also requires enhanced disclosures about revenue, provides guidance for transactions that were not previously addressed comprehensively, and improves guidance for multiple-element arrangements. The guidance was effective for public companies for annual periods beginning after December 15, 2017 as well as interim periods within those annual periods using either the full retrospective approach or modified retrospective approach. The Company adopted the new standard effective July 1, 2018 using the modified retrospective approach. Refer to Note 6 for the impact of adoption.

#### **Share-Based Compensation**

In May 2017, the FASB issued Accounting Standards Update ( ASU ) No. 2017-09, *Scope of Modification Accounting*, which amends ASC Topic 718, *Compensation - Stock Compensation*. The amendments in this Update provide guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The amendments are effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years and early adoption is permitted. The Company has adopted this standard in the first quarter of fiscal 2019 and it did not have a material effect on its financial statements.





*To be adopted in future periods*

### Leases

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* in order to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet for those leases previously classified as operating leases under GAAP. The standard requires, in most instances, a lessee to recognize on its balance sheet a liability to make lease payments (the lease liability) and also a right-of-use asset representing its right to use the underlying asset for the lease term. The amendments are effective for fiscal years beginning after December 15, 2018, including interim periods within those periods, using a modified retrospective approach and early adoption is permitted. The Company is currently in the process of evaluating the impact of adoption of this standard on its financial statements.

### Share-Based Compensation

In June 2018, the FASB issued ASU No. 2018-07, *Compensation Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*. The new standard aligns the measurement and classification guidance for share-based payments to nonemployees with the guidance for share-based payments to employees, with certain exceptions. Under the guidance, the measurement of equity-classified nonemployee awards will be fixed at the grant date, which may lower their cost and reduce volatility in the income statement. The standard will be effective for the Company on July 1, 2020. Early adoption is permitted. The Company is currently in the process of evaluating the impact of adoption of this standard on its financial statements.

### Fair Value Measurement

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework Changes to the Disclosure Requirements for Fair Value Measurement*. The new standard eliminates, adds and modifies certain disclosure requirements for fair value measurement as part of its disclosure framework project. The amount and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy will no longer be required to be disclosed, but public companies will be required to disclose the range and weighted average used to develop significant unobservable inputs for Level 3 fair value measurements. This standard will be effective for the Company on July 1, 2020. Early adoption is permitted. The adoption of this guidance is not expected to have a significant impact on the Company's financial statements.

**3. Share-based Compensation Plans:**

The Company uses stock options and awards of restricted stock to provide long-term incentives for its employees, non-employee directors and certain consultants. The Company has two equity compensation plans under which awards are currently authorized for issuance, the 2013 Employee Stock Purchase Plan and the 2013 Equity and Incentive Plan. No awards have been issued to date under the 2013 Employee Stock Purchase Plan and all of the 128,571 shares previously authorized under this plan remain available for issuance. A summary of the stock option and restricted stock activity for the six months ended December 31, 2018 and 2017 is as follows:

|  | <b>For the Six Months Ended December 31,</b> |  |               |  |
|--|--|--|---------------|--|
|  | <b>2018</b>                                  |  | <b>2017</b>   |  |
|  | <b>Shares</b>                                | <b>Weighted<br/>Average<br/>Exercise<br/>Price</b> | <b>Shares</b> | <b>Weighted<br/>Average<br/>Exercise<br/>Price</b> |
| (In thousands, except per share amounts)                         |  |  |               |  |
| Outstanding at June 30,  | 3,107  | \$ 10.93   | 2,714         | \$ 12.96   |
| Granted  | 983  | 4.59   | 746           | 4.83   |
| Exercised  | (29)   | 4.52   | (7)           | 0.35   |
| Forfeited  | (213)  | 7.41   | (309)         | 9.76   |
| Expired  | (184)  | 13.40  | (15)          | 15.35  |
| Outstanding at December 31,                                      | 3,664  | \$ 9.37  | 3,129         | \$ 11.35   |
| Exercisable at December 31,                                      | 2,034  |  | 1,694         |  |
| Weighted average fair value of options granted during the period | \$ 2.38                                      |  | \$ 3.49       |  |

For the three and six months ended December 31, 2018, share-based compensation expense related to stock options and restricted stock awarded to employees, non-employee directors and consultants amounted to approximately \$1.1 million and \$2.3 million, respectively, compared to \$1.3 million and \$2.8 million, respectively, for the three and six months ended December 31, 2017.

As of December 31, 2018, there was \$6.3 million of unrecognized compensation expense related to non-vested stock options and restricted stock. During the six months ended December 31, 2018, 987,000 stock options and restricted stock awards were granted to the Company's employees and non-employee directors under the 2013 Equity and Incentive Plan. The fair value of each option granted is estimated on the grant date using the Black-Scholes stock option pricing model. The following assumptions were made in estimating fair value:

| <b>Assumption</b>       | <b>Six months ended<br/>December 31, 2018</b> |            |
|-------------------------|---|------------|
| Dividend yield          | 0.00%   |            |
| Expected term           | 6.00  | 6.25 years |
| Risk-free interest rate | 2.76  | 3.11%      |
| Expected Volatility     | 69.22%  |            |



**4. Investments:**

Cash in excess of immediate requirements is invested in accordance with the Company's investment policy that primarily seeks to maintain adequate liquidity and preserve capital.

The following table summarizes the Company's investments by category as of December 31, 2018 and June 30, 2018:

| In thousands                     | December 31,<br>2018 | June 30,<br>2018 |
|----------------------------------|----------------------|------------------|
| <u>Investments Current:</u>      |                      |                  |
| Certificates of deposit          | \$                   | \$ 2,106         |
| Debt securities held-to-maturity | 72,097               | 71,734           |
| Total investments current        | \$ 72,097            | \$ 73,840        |

A summary of the Company's debt securities classified as held-to-maturity is as follows:

| In thousands                           | At December 31, 2018 |                              |                               | Fair<br>Value |
|--|----------------------|------------------------------|-------------------------------|---------------|
|  | Amortized<br>Cost    | Gross<br>Unrealized<br>Gains | Gross<br>Unrealized<br>Losses |               |
| <u>Investments Current:</u>            |                      |                              |                               |               |
| U.S. government and agency obligations | \$ 72,097            | \$                           | \$ (41)                       | \$ 72,056     |
| Total investments current              | \$ 72,097            | \$                           | \$ (41)                       | \$ 72,056     |

| In thousands                           | At June 30, 2018  |                              |                               | Fair<br>Value |
|--|-------------------|------------------------------|-------------------------------|---------------|
|  | Amortized<br>Cost | Gross<br>Unrealized<br>Gains | Gross<br>Unrealized<br>Losses |               |
| <u>Investments Current:</u>            |                   |                              |                               |               |
| U.S. government and agency obligations | \$ 69,731         | \$                           | \$ (60)                       | \$ 69,671     |
| Corporate obligations                  | 2,003             |                              | (1)                           | 2,002         |
|  | \$ 71,734         | \$                           | \$ (61)                       | \$ 71,673     |

The amortized cost and fair value of held-to-maturity debt securities as of December 31, 2018, by contractual maturity, were as follows:

| In thousands            | <b>Amortized<br/>Cost</b> | <b>Fair<br/>Value</b> |
|-------------------------|---------------------------|-----------------------|
| Due in one year or less | \$ 72,097                 | \$ 72,056             |
|                         | \$ 72,097                 | \$ 72,056             |

The Company believes that the unrealized losses disclosed above were primarily driven by interest rate changes rather than by unfavorable changes in the credit ratings associated with these securities and as a result, the Company continues to expect to collect the principal and interest due on its debt securities that have an amortized cost in excess of fair value. At each reporting period, the Company evaluates securities for impairment when the fair value of the investment is less than its amortized cost. The Company evaluated the underlying credit quality and credit ratings of the issuers, noting neither a significant deterioration since purchase nor other factors leading to an other-than-temporary impairment. Therefore, the Company believes these losses to be temporary. As of December 31, 2018, the Company did not have the intent to sell any of the securities that were in an unrealized loss position at that date.

##### **5. Fair Value of Financial Instruments and Investments:**

Certain assets and liabilities are measured at fair value in the Company's financial statements or have fair values disclosed in the notes to the financial statements. These assets and liabilities are classified into one of three levels of a hierarchy defined by GAAP. The Company's assessment of the significance of a particular item to the fair value measurement in its entirety requires judgment, including the consideration of inputs specific to the asset or liability.

The following methods and assumptions were used to estimate the fair value and determine the fair value hierarchy classification of each class of financial instrument included in the table below.

**Cash and Cash Equivalents.** The carrying value of cash and cash equivalents approximates fair value as maturities are less than three months.

**Certificates of Deposit.** The Company's certificates of deposit are placed through an account registry service. The fair value measurement of the Company's certificates of deposit is considered Level 2 of the fair value hierarchy as the inputs are based on quoted prices for identical assets in markets that are not active. The carrying amounts of the Company's certificates of deposit reported in the Unaudited Condensed Balance Sheets approximate fair value.

**Debt securities held-to-maturity.** The Company's investments in debt securities classified as held-to-maturity generally include U.S. Treasury Securities, government agency obligations, and corporate obligations. U.S. Treasury Securities are valued using quoted market prices. Valuation adjustments are not applied. Accordingly, U.S. Treasury Securities are considered Level 1 of the fair value hierarchy. The fair values of U.S. government agency obligations and corporate obligations are generally determined using recently executed transactions, broker quotes, market price quotations where these are available or other observable market inputs for the same or similar securities. As such, the Company classifies its investments in U.S. government agency obligations and corporate obligations within Level 1 or Level 2 of the hierarchy, depending on the information used to determine the fair values.



The following fair value hierarchy table presents information about each major category of the Company's financial assets and liabilities measured at fair value on a recurring basis:

| 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<br>Fair<br>Value | Total<br>Carrying<br>Value |
|--|---|---|--|------------------------|----------------------------|
| <b>December 31, 2018</b>               |   |   |  |                        |                            |
| Cash and cash equivalents              | \$ 23,978   | \$  | \$   | \$ 23,978              | \$ 23,978                  |
| Held-to-maturity investments:          |   |   |  |                        |                            |
| U.S. government and agency obligations | 72,056  |   |  | 72,056                 | 72,097                     |
| <b>Total assets</b>                    | <b>\$ 96,034</b>                                      | <b>\$</b>   | <b>\$</b>  | <b>\$ 96,034</b>       | <b>\$ 96,075</b>           |
| <b>June 30, 2018</b>                   |   |   |  |                        |                            |
| Cash and cash equivalents              | \$ 31,065   | \$  | \$   | \$ 31,065              | \$ 31,065                  |
| Certificates of deposit                |   | 2,100   |  | 2,100                  | 2,106                      |
| Held-to-maturity investments:          |   |   |  |                        |                            |
| Corporate obligations                  |   | 2,002   |  | 2,002                  | 2,003                      |
| U.S. government and agency obligations | 69,671  |   |  | 69,671                 | 69,731                     |
| <b>Total assets</b>                    | <b>\$ 100,736</b>                                     | <b>\$ 4,102</b>   | <b>\$</b>  | <b>\$ 104,838</b>      | <b>\$ 104,905</b>          |

## 6. Collaboration Agreements

### *Biogen*

On July 1, 2015, the Company entered into a Collaboration Agreement (the "Collaboration Agreement"), a Manufacturing License and Technology Transfer Agreement (the "Manufacturing Agreement"), and the Common Stock Purchase Agreement (the "Equity Agreement") with Biogen (collectively, the "Biogen Agreement"), pursuant to which the Company and Biogen will collaborate to develop, seek regulatory approval for and commercialize gene therapy products to treat XLRs, XLRP, and discovery programs targeting three indications based on the Company's adeno-associated virus vector technologies.

Under the Collaboration Agreement, the Company has granted to Biogen with respect to the XLRs and XLRP programs, and upon exercise of the option for the applicable discovery program, an exclusive, royalty-bearing license, with the right to grant sublicenses, to use adeno-associated virus vector technology and other technology controlled by the Company for the licensed products or discovery programs developed under the Collaboration Agreements. Biogen and the Company have also granted each other worldwide licenses, with the right to grant sublicenses, of their respective interests in other intellectual property developed under the collaboration outside the licensed products or discovery programs. Biogen has pre-funded the Company to conduct all development activities through the completion of a first in human trial for the XLRs program and all development activities through the date that the Investigational New Drug Application (IND) becomes effective and the completion of a natural history study for the XLRP program. In addition, Biogen has pre-funded the Company to conduct discovery, research and development

activities for additional drug candidates through the stage of clinical candidate designation for discovery programs targeting three indications (of which one indication has two development plans at contract inception), after which, Biogen may exercise an option to continue to develop, seek regulatory approval for and commercialize the designated clinical candidate. The pre-funded research and development activities for each program are referred to as Pre-Funded Activities . Biogen will reimburse the Company on an FTE basis for any additional expenses related to development activities that the Company incurs ( Post-Funded Activities ).



In February 2016, the Company announced Biogen's selection of adrenoleukodystrophy as the non-ophthalmic indication of the discovery programs. Under the terms of the Collaboration Agreement, the Company, in part through its participation in joint committees with Biogen, will participate in overseeing the development and commercialization of these specific programs.

Pursuant to the Manufacturing Agreement, Biogen may elect an option to receive a manufacturing license for up to six genes for a fixed fee per gene elected. If exercised, the Company becomes eligible to receive certain event milestones and royalties.

Under the Collaboration Agreement, the Company was paid an upfront nonrefundable fee of \$94.0 million of which \$58.4 million was contractually described as relating to the Pre-Funded Activities ( Pre-Funded Amounts ) and \$35.6 million was contractually described as relating to the access of licenses. In addition, under the terms of the Equity Agreement, Biogen purchased 1,453,957 shares of the Company's common stock at a price of \$20.63 per share, for an aggregate cash purchase price of \$30.0 million of which \$10.8 million was considered to be allocated consideration as part of the Collaboration Agreement. The shares issued to Biogen represented approximately 8.1% of the Company's outstanding common stock on a post-issuance basis, calculated on the number of shares that were outstanding at June 30, 2015, and constitute restricted securities that may not be resold by Biogen other than in a transaction registered under, or pursuant to an exemption from the registration requirements of, the Securities Act of 1933, as amended.

The Company is also eligible to receive total payments of up to \$472.5 million based on the successful achievement of future milestones under its XLRS and XLRP programs. For XLRS, the Company is eligible to receive up to: (i) \$45.0 million in milestone payments based upon the successful achievement of clinical milestones (relating to dosing in specified trials), (ii) \$155.0 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories and (iii) \$65.0 million in milestone payments based upon the achievement of worldwide sales targets. For the XLRS program, the Company has an option to share development costs and profits after the initial clinical trial data are available instead of receiving milestone payments. For XLRP, the Company is eligible to receive up to: (i) \$42.5 million in milestone payments based upon successful achievement of clinical milestones (relating to dosing in specified trials), (ii) \$102.5 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories and (iii) \$62.5 million in milestone payments based upon the achievement of worldwide sales targets. For the XLRP program, the Company has an option to share development costs and profits after the initial clinical trial data are available instead of receiving milestone payments. In addition, the Company is eligible to receive payments of up to \$592.5 million based on the exercise of the option for and the successful achievement of future milestones under its discovery programs. Each discovery program is categorized as Category A, Category B or Category C depending on the nature of the indication it seeks to address. For Category A, the Company is eligible to receive payments of up to: (i) \$20.0 million based upon the successful achievement of clinical milestones (relating to dosing in specified trials) and (ii) \$70.0 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories. For Category B, the Company is eligible to receive payments of up to: (i) \$27.5 million based upon the successful achievement of clinical milestones (relating to dosing in specified trials) and (ii) \$105.0 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories. For Category C, the Company is eligible to receive payments of up to: (i) \$40.0 million based upon the successful achievement of clinical milestones (relating to dosing in specified trials) and (ii) \$140.0 million in milestone payments based upon the achievement of regulatory approvals and first commercial sale in specified territories. Under certain limited circumstances, if there are discovery products from more than one discovery program in any of Category A, Category B or Category C, then the milestone payments under the applicable category shall be payable for the applicable discovery product from each such discovery program to achieve the specified milestones.

Biogen will also pay revenue-based royalties for each licensed product at tiered rates ranging from high-single digit to mid-teen percentages of annual net sales of the XLRS or XLRP products and at rates ranging from mid-single digit to

low-teen percentages of annual net sales for the discovery products.

Prior to 2018, the Company received a \$5.0 million milestone payment related to initial dosing of a XLRS patient. In April 2018, the Company triggered a \$2.5 million milestone payment related to the initial dosing of a XLRP patient. In July 2018, the Company triggered a \$10.0 million milestone payment related to the treatment of a first patient of second cohort in a Phase 1/2 Clinical XLRP Study.

On December 7, 2018, the Company received notice from Biogen that Biogen had elected to terminate the Collaboration Agreement effective as of March 8, 2019. While the Company will recognize additional revenue as it continues to perform under that agreement prior to the effective date of the termination of the Collaboration Agreement, the Company will not receive any milestone-based or royalty payments under that agreement after its termination.

#### *Accounting Analysis*

For the periods prior to July 1, 2018, the Company applied the provisions of ASC 605 in accounting for this arrangement. Refer to the Company's Annual Report on Form 10-K for the year ended June 30, 2018, filed with the Securities and Exchange Commission on September 11, 2018, for the accounting analysis under these provisions.

Under ASC 605 and Topic 606, the Company has concluded that the Collaboration Agreement, the Manufacturing Agreement and the Equity Agreement should be accounted for as one arrangement as the agreements were with the same party and were negotiated and executed contemporaneously.

The performance obligations and the allocated transaction price as of the date of initial application of Topic 606 are as follows (in thousands):

| <b>Performance Obligations:</b>                              | <b>Allocated<br/>Transaction Price</b> |
|--|--|
| XLRS License and Pre-Funded Activities                       | \$ 52,060                              |
| XLRP License and Pre-Funded Activities                       | 43,570                                 |
| Pre-Funded Activities associated with the Discovery Programs | 16,700                                 |
|  | \$ 112,330                             |

The Pre-Funded Activities associated with the Discovery Programs amount is comprised of four distinct performance obligations based on the separate development plans for discovery candidates at contract inception. The Company concluded that the delivered license was not distinct from the Pre-Funded Activities as Biogen cannot obtain the benefit of the license without the related services. Further, each of the license and related Pre-Funded Activities performance obligation is considered a distinct performance obligation as each development plan is pursued independent of every other development plan.

The Company concluded that Post-Funded Activities represent customer options that are not material rights as any services requested by Biogen and provided by the Company are reimbursed at a rate that reflects the estimated standalone selling price for the services. As such, the Company will recognize revenue related to Post-Funded Activities as the services are provided. Through the date of adoption of ASC 606, the Company has recognized revenue of \$4.7 million for Post-Funded Activities. The Company recorded revenue of \$0.9 million and \$2.0 million in the three and six months period ended December 31, 2018 related to Post-Funded Activities.

The Company concluded that the option to receive i) commercial licenses for the Discovery Programs that achieve clinical candidate designation, as defined in the Collaboration Agreement and ii) manufacturing licenses for up to six genes pursuant to the Manufacturing Agreement represent customer options that are not material rights as the exercise price for such options reflects the estimated standalone selling price for such option. As such, the Company will account for such option if and when the options are exercised.

As of the date of the initial application of Topic 606, the total transaction price for the Biogen Agreement was \$112.3 million which included a \$5.0 million milestone payment for initiation of dosing of XLRS and a \$2.5 million milestone payment for initiation of dosing of XLRP. The Company used the most-likely method to determine the amount of variable consideration in the Biogen Agreement. The Company believes that any estimated amount of variable consideration related to clinical and regulatory milestone payments should be fully constrained as the achievement of such milestones is highly susceptible to factors outside of the Company's control. The Company has determined that the commercial

milestones and sales-based royalties will be recognized when the related sales occur as they were deemed to relate predominately to the license granted and therefore have also been excluded from the transaction price. The Company will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

In the quarter ended September 30, 2018, the Company received a \$10.0 million milestone payment related to XLRP which increased the transaction price. Based on an understanding between the parties in the quarter ended September 30, 2018, the Company also reallocated \$1.1 million of Pre-Funded Amounts to cover Post-Funded Activities which resulted in a decrease to the transaction price and deferred revenue of \$1.1 million in the quarter ended September 30, 2018. Additionally, the Company reallocated \$1.8 million of variable consideration between Pre-Funded Activities associated with Discovery program performance obligations based on changes to the underlying development plans of the product candidates. The total transaction price did not change in the quarter ended December 31, 2018.

The reallocation between Discovery Programs generated an insignificant cumulative catch up adjustment to revenue in the quarter ended September 30, 2018. The cumulative catch-up adjustment to revenue that relate to changes or reallocations of the transaction price are further discussed in the *Summary of Contract Assets and Liabilities* section below.

The transaction price was allocated to the performance obligations based on the relative estimated standalone selling price of each performance obligation or, in the case of certain variable consideration, to one or more performance obligations. The estimated standalone selling prices for performance obligations, that include a license and Pre-Funded Activities, were developed using the estimated selling price of the license and an estimate of the overall effort to perform the Pre-Funded Activities. The estimated selling price of the licenses were determined using a discounted cash flow valuation utilizing forecasted revenues and costs for the Company's product candidate licenses.

The Company will recognize revenue related to the performance obligations which include a license and Pre-Funded Activities over the estimated period of the research and development services using a proportional performance model. The Company measures proportional performance based on the costs incurred relative to the total costs expected to be incurred to satisfy the performance obligation. Management believes that recognizing revenue on a proportional performance basis based on costs incurred faithfully depicts the transfer of goods and services to the customer because the customer consumes the Company's services as such services are performed. The Company will account for the termination of the Collaboration Agreement upon the effective date of the termination. The Company updated its total costs estimated to be incurred to satisfy the performance obligations as of December 31, 2018; however, such estimates do not include any impact of the termination which is expected to occur in the three-month period ended March 31, 2019.

During the three and six months ended December 31, 2018 and 2017, the Company recorded revenue of \$5.9 million and \$19.9 million, and \$4.8 million and \$15.1 million, respectively, related to its efforts under the Biogen Agreement. The Company has net accounts receivable balances with Biogen as of December 31, 2018 and June 30, 2018 of \$0.9 million and \$1.7 million, respectively, related to the Biogen Agreement. As of December 31, 2018, the Company had recorded \$20.4 million in deferred revenue related to the Biogen Agreement that will be recognized over the remaining performance period. Absent the termination of the Collaboration Agreement, the Company expects to satisfy its remaining performance obligations under the Biogen Agreement within the next three years. If the Collaboration Agreement is terminated effective March 8, 2019, any remaining deferred revenue will be recognized as revenue.

The company's revenue is comprised of the following related to the Biogen Agreement:

| In thousands,                      | For the Three<br>Months Ended |                 | For the Six Months<br>Ended |                  |
|------------------------------------|-------------------------------|-----------------|-----------------------------|------------------|
|                                    | December 31,<br>2018          | 2017            | December 31,<br>2018        | 2017             |
| Collaboration revenue              |                               |                 |                             |                  |
| Licenses and related services      | \$ 4,362                      | \$ 3,735        | \$ 8,981                    | \$ 13,489        |
| Development services               | 903                           | 1,096           | 1,961                       | 1,650            |
| Milestone revenue                  | 630                           |                 | 8,978                       |                  |
| <b>Total collaboration revenue</b> | <b>\$ 5,895</b>               | <b>\$ 4,831</b> | <b>19,920</b>               | <b>\$ 15,139</b> |

License and related services revenue is comprised of revenue related to the Company's completion of performance obligations that contain the delivery of licenses and Pre-Funded Activities. Development services revenue relates to the delivery of Post Funded Activities. Milestone revenue relates to the portion of milestone payments received that are recognized as revenue based on the proportional performance of the underlying performance obligation.

#### *Summary of Contract Assets and Liabilities*

The following table presents changes in the balances of our contract assets and liabilities during the three and six months ended December 31, 2018 (in thousands):

|   | Balance at<br>Beginning<br>of Period | Additions | Deductions | Balance at<br>End of<br>Period |
|---|--------------------------------------|-----------|------------|--------------------------------|
| <b>Three months ended December 31, 2018</b> |                                      |           |            |                                |
| Contract assets                             | \$                                   | \$        | \$         | \$                             |
| Contract liabilities:                       |                                      |           |            |                                |
| Deferred revenue                            | \$ 25,425                            | \$        | \$ 4,993   | \$ 20,432                      |
| <b>Six months ended December 31, 2018</b>   |                                      |           |            |                                |
| Contract assets                             | \$                                   | \$        | \$         | \$                             |
| Contract liabilities:                       |                                      |           |            |                                |
| Deferred revenue                            | \$ 29,521                            | \$ 10,000 | \$ 19,089  | \$ 20,432                      |

The Company recorded an entry to increase deferred revenue and accumulated deficit for \$22.6 million as of July 1, 2018 related to the adoption of Topic 606. The impact of the adoption of Topic 606 is reflected within the beginning of period balance for the six months ended December 31, 2018. Additions for the six months ended December 31, 2018 include the \$10 million milestone payment received associated with the XLRP program. Deductions from deferred revenue for the three months ended December 31, 2018 represent revenue recognized related to the deferred revenue balance at the beginning of the period. Deductions from deferred revenue for the six months ended December 31, 2018 include revenue recognized related to the deferred revenue balance of \$9.6 million, revenue

recognized related to additions to deferred revenue of \$8.3 million (of which \$7.8 million relates to performance in prior quarters) and \$1.1 million of variable consideration that was constrained.

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## ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis provides an overview of our financial condition as of December 31, 2018, and results of operations for the three and six months ended December 31, 2018 and 2017. This discussion should be read in conjunction with the accompanying Unaudited Condensed Financial Statements and accompanying notes, as well as our Annual Report on Form 10-K for the year ended June 30, 2018, ( June 2018 Form 10-K ). In addition to historical financial information, the following discussion contains forward-looking statements that reflect our plans, estimates, assumptions and beliefs. Our actual results could differ materially from those discussed in the forward-looking statements. Factors that could cause or contribute to these differences include those discussed below and elsewhere in this report as well as those set forth in Part I, Item 1A, Risk Factors of the June 2018 Form 10-K. Forward-looking statements include information concerning our possible or assumed future results of operations, business strategies and operations, financing plans, potential growth opportunities, potential market opportunities and the effects of competition. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as anticipates, believes, could, seeks, estimates, expects, intends, may, plans, predicts, projects, should, will, would or similar expressions and the negatives of those terms. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our management's plans, estimates, assumptions and beliefs only as of the date of this report. Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

As used herein, except as otherwise indicated by context, references to we, us, our, or the Company refer to Applied Genetic Technologies Corporation.

### Overview

We are a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases. Our initial focus is in the field of ophthalmology, where we have active clinical programs in X-linked retinoschisis (XLRS), achromatopsia (ACHM) and X-linked retinitis pigmentosa (XLRP), and a preclinical program in optogenetics. In addition to ophthalmology, we have initiated preclinical programs in adrenoleukodystrophy (ALD) and otology. With a number of important clinical milestones on the horizon, we believe we are well positioned to advance multiple programs towards pivotal studies. In addition to our product pipeline, we have also developed broad technological capabilities through our collaborations with Synpromics Limited (Synpromics) and the University of Florida, which provide us with expertise in vector design and manufacturing as well as synthetic promoter development and optimization.

Since our inception in 1999, we have devoted substantially all of our resources to development efforts relating to our proof-of-concept programs in ophthalmology and alpha-1 antitrypsin deficiency, or AAT deficiency, an inherited orphan lung disease, including activities to manufacture product in compliance with good manufacturing practices, preparing to conduct and conducting clinical trials of our product candidates, providing general and administrative support for these operations and protecting our intellectual property. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations to date primarily through public offering of our common stock, private placement of preferred stock, and collaborations. We have also been the recipient, either independently or with our collaborators, of grant funding administered through federal, state, and local governments and agencies, including the United States Food and Drug Administration, or FDA, and by patient advocacy groups such as The Foundation Fighting Blindness, or FFB, and the Alpha-1 Foundation.

We have incurred losses from operations in each year since inception except for fiscal 2017. Our net loss for the six-month period ended December 31, 2018 was \$3.0 million while the net loss for the fiscal year ended June 30, 2018 was \$21.3 million, compared to net income of \$0.4 million and net loss of \$1.4 million for each of the fiscal years



ended June 30, 2017 and 2016, respectively. Substantially all our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant operating expenses for at least the next several years and anticipate that such expenses will increase substantially in connection with our ongoing activities, as we:

conduct preclinical studies and clinical trials for our XLRS, ACHM and XLRP product candidates;

continue our research and development efforts, including exploration through early preclinical studies of potential applications of our gene therapy platform in:

orphan ophthalmology indications;

non-orphan ophthalmology indications including wet AMD and other inherited retinal diseases; and

other inherited diseases, such as otology and CNS indications.

manufacture clinical trial materials and develop larger-scale manufacturing capabilities;

seek regulatory approval for our product candidates;

further develop our gene therapy platform;

add personnel to support our collaboration, product development and commercialization efforts; and

continue to operate as a public company.

As of December 31, 2018, we had cash and cash equivalents and investments totaling \$96.1 million.

We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years and which we believe is subject to significant uncertainty. We believe that our existing cash and cash equivalents and investments at December 31, 2018, will be sufficient to allow us to generate data from our ongoing clinical programs, to move our pre-clinical optogenetic program in collaboration with Bionic Sight into the clinic and to fund our currently planned research and discovery programs for at least the next two years. In order to complete the process of obtaining regulatory approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding. Also, our current operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our products.

On December 7, 2018, we received written notice from Biogen MA Inc. (Biogen), a wholly owned subsidiary of Biogen Inc., that Biogen has elected to terminate the Collaboration and License Agreement between Biogen and us effective as of March 8, 2019. Upon expected termination of the Collaboration Agreement, we will receive back the exclusive license rights to develop, manufacture and commercialize the product candidates for all of our partnered programs including our XLRP program, XLRS program and our three discovery programs. As we reported on December 12, 2018, based on topline interim six-month data from our Phase 1/2 clinical trial of our XLRS product

candidate that showed no clinical activity at six-months, we will complete patient monitoring activities on the XLRS program according to the clinical protocol, but we will not further develop our XLRS product candidate. We plan to continue to advance our XLRP and ACHM product candidates, as we believe the general safety and tolerability of our gene delivery platform is supported by our XLRS clinical data, and we are evaluating next steps for our discovery programs.

### **Critical Accounting Policies**

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and share-based compensation. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

For a description of our accounting policies that, in our opinion, involve the most significant application of judgment or involve complex estimation and which could, if different judgments or estimates were made, materially affect our reported results of operations, see Management's Discussion and Analysis of Financial Condition and Results of Operations Critical Accounting Policies and Estimates in our June 2018 Form 10-K, for the year ended June 30, 2018.

The Company adopted Topic 606 on July 1, 2018. See Note 2 to these Unaudited Condensed Financial Statements included in this Quarterly Report on Form 10-Q for a description of our revenue recognition accounting policies.

### **New Accounting Pronouncements**

Refer to Note 2 to these Unaudited Condensed Financial Statements included in this quarterly report for further information on recently issued accounting standards.

### **Financial operations review**

#### *Revenue*

We primarily generate revenue through collaboration agreements, sponsored research arrangements with nonprofit organizations for the development and commercialization of product candidates and from federal research and development grant programs. On December 7, 2018, the Company received a letter from Biogen that served as notice that Biogen had elected to terminate the Collaboration Agreement in its entirety effective as of March 8, 2019. We will account for the termination of the Collaboration Agreement upon the effective date of the termination. If the Collaboration Agreement is terminated effective March 8, 2019, deferred revenue as of December 31, 2018 of \$20.4 million will be recognized as revenue in the three month period ending March 31, 2019. Thereafter, no more collaboration revenue related to the Collaboration Agreement will be recognized. In the future, we may generate revenue from a combination of product sales, license fees, milestone payments, development services, research and development grants, and from collaboration and royalty payments for the sales of products developed under licenses of our intellectual property. We do not expect to generate revenue from product sales for the foreseeable future, if at all. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenue, our results of operations and financial position would be materially adversely affected.

#### *Research and development expenses*

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

employee-related expenses, including salaries, benefits, travel and share-based compensation expense;

expenses incurred under agreements with academic research centers, contract research organizations, or CROs, and investigative sites that conduct our clinical trials;

license and sublicense fees and collaboration expenses;

the cost of acquiring, developing, and manufacturing clinical trial materials; and

facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supplies.

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites.

We cannot determine with certainty the duration and completion costs of the current or future clinical trials of our product candidates or if, when, or to what extent we will generate revenues from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs, and timing of clinical trials and development of our product candidates will depend on a variety of factors, including:

the scope, rate of progress, and expense of our ongoing as well as any additional clinical trials and other research and development activities;

our ability to enter into partnership or collaboration agreements with third parties;

the timing and level of activity as determined by us or jointly with our partners;

the level of funding received from our partners;

whether or not we elect to cost share with our partners;

the countries in which trials are conducted;

future clinical trial results;

uncertainties in clinical trial enrollment rates or drop-out or discontinuation rates of patients;

potential additional safety monitoring or other studies requested by regulatory agencies or elected as best practice by us;

increased cost and delay associated with manufacturing or testing issues, including ongoing quality assurance, qualifying new vendors and developing in-house capabilities;

significant and changing government regulation; and

the timing and receipt of any regulatory approvals.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate or if we experience significant delays in enrollment in or execution of any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

From inception through December 31, 2018, we have incurred approximately \$186.9 million in research and development expenses. We expect our research and development expenses to increase for the foreseeable future as we continue the development of our product candidates and explore potential applications of our gene therapy platform in other indications.

#### *General and administrative expenses*

General and administrative expenses consist primarily of salaries and related costs for personnel, including share-based compensation and travel expenses for our employees in executive, operational, legal, business development, finance and human resource functions. Other general and administrative expenses include costs to support employee training and development, board of directors costs, depreciation, insurance expenses, facility-related costs not otherwise included in research and development expense, professional fees for legal services, including patent-related expenses, and accounting, investor relations, corporate communications and information technology services. We anticipate that our general and administrative expenses will continue to increase in the future as we hire additional employees to support our continued research and development efforts, collaboration arrangements, and the potential commercialization of our product candidates. Additionally, if and when we believe a regulatory approval of the first product candidate appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidates.

*Other income (expense), net*

Other income and expense consists primarily of interest earned on cash and cash equivalents and our held-to-maturity investments.

*Provision for income taxes*

Income tax expense (benefit) for the three and six months ended December 31, 2018 was \$19,000 and \$38,000, respectively, compared to (\$791,000) for both the three and six months ended December 31, 2017. The income tax expense for the three and six months ended December 31, 2018, was primarily driven by interest expense related to the Company's uncertain tax position.

## Results of Operations

### Comparison of the three months ended December 31, 2018 to the three months ended December 31, 2017

#### Revenue

|                              | For the Three Months       |          | Increase<br>(Decrease) | % Increase<br>(Decrease) |
|------------------------------|----------------------------|----------|------------------------|--------------------------|
|                              | Ended December 31,<br>2018 | 2017     |                        |                          |
|                              | (dollars in thousands)     |          |                        |                          |
| Collaboration revenue        |                            |          |                        |                          |
| License and related services | \$ 4,362                   | \$ 3,735 | \$ 627                 | 17%                      |
| Development services         | 903                        | 1,096    | (193)                  | (18)%                    |
| Milestone revenue            | 630                        |          | 630                    | nm%                      |
| Total collaboration revenue  | 5,895                      | 4,831    | 1,064                  | 22%                      |
| Grant revenue                | 39                         | 21       | 18                     | 86%                      |
| Total revenue                | \$ 5,934                   | \$ 4,852 | \$ 1,082               | 22%                      |

Total revenue for the three months ended December 31, 2018 was \$5.9 million, compared to \$4.9 million for the three months ended December 31, 2017. The increase was primarily due to increased license and related services revenue of \$0.6 million and an increase in milestone revenue of \$0.6 million, partially offset by decreased development services revenue of \$0.2 million. Effective July 1, 2018, the Company adopted Topic 606. Based on the Company's Topic 606 revenue recognition methodology, milestone payments are recognized based on the proportional performance of the underlying performance obligation for which the milestone payment relates. The decrease in development services revenue was primarily due to timing of Phase 1/2 study activities related to the Company's XLRP program. The increase in license and related service revenue was due to the Company's revised pattern of revenue recognition under Topic 606. For the three months ended December 31, 2018, license and related services, which includes the Pre-Funded Activities associated with each program under the Biogen Collaboration, are recognized on a proportional performance basis under Topic 606. For the three months ended December 31, 2017, license and related services revenue was recognized based on a straight-line basis over the estimated service period under the Company's prior revenue recognition methodology.

#### Research and development expense

The following table summarizes our research and development expenses by product candidate or program for the three months ended December 31, 2018 and 2017:

|  | For the Three Months       |        | Increase<br>(Decrease) | % Increase<br>(Decrease) |
|--|----------------------------|--------|------------------------|--------------------------|
|  | Ended December 31,<br>2018 | 2017   |                        |                          |
|  | (dollars in thousands)     |        |                        |                          |
| External research and development expenses |                            |        |                        |                          |
| ACHM                                       | \$ 955                     | \$ 675 | \$ 280                 | 41%                      |
| XLRS                                       | 433                        | 520    | (87)                   | (17)%                    |



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|  |          |          |          |       |
|--|----------|----------|----------|-------|
| XLRP   | 336      | 547      | (211)    | (39)% |
| Research and discovery programs                  | 1,158    | 1,969    | (811)    | (41)% |
| Total external research and development expenses | 2,882    | 3,711    | (829)    | (22)% |
| Internal research and development expenses       |          |          |          |       |
| Employee-related costs                           | 2,624    | 2,092    | 532      | 25%   |
| Share-based compensation                         | 541      | 647      | (106)    | (16)% |
| Other  | 1,536    | 1,276    | 260      | 20%   |
| Total internal research and development expenses | 4,701    | 4,015    | 686      | 17%   |
| Total research and development expense           | \$ 7,583 | \$ 7,726 | \$ (143) | (2)%  |

External research and development costs consist of collaboration, licensing, manufacturing, testing, and other miscellaneous expenses that are directly attributable to our most advanced product candidates and discovery programs. We do not allocate personnel-related costs, including stock-based compensation, costs associated with broad technology platform improvements or other indirect costs, to specific programs, as they are deployed across multiple projects under development and, as such, are separately classified as internal research and development expenses in the table above.

Research and development expenses for the three months ended December 31, 2018 were \$7.6 million, compared to \$7.7 million for the three months ended December 31, 2017, a decrease of \$0.1 million, or 2%. This decrease was primarily due to decreased external spending of \$0.8 million, partially offset by increased internal spending of \$0.7 million. The decrease in external spending was primarily due to decreased research and discovery spending associated with our pre-clinical otology and ophthalmology programs. The increase in internal spending was primarily due to hiring of additional employees to support clinical trial execution and research and development activities.

#### *General and administrative expense*

|   | <b>For the Three Months</b>        |                 | <b>Increase<br/>(Decrease)</b> | <b>% Increase<br/>(Decrease)</b> |
|---|------------------------------------|-----------------|--------------------------------|----------------------------------|
|   | <b>Ended December 31,<br/>2018</b> | <b>2017</b>     |                                |                                  |
|   | <b>(dollars in thousands)</b>      |                 |                                |                                  |
| Employee-related costs                          | \$ 1,046                           | \$ 1,388        | \$ (342)                       | (25)%                            |
| Share-based compensation                        | 553                                | 685             | (132)                          | (19)%                            |
| Legal and professional fees                     | 82                                 | 73              | 9                              | 12%                              |
| Other   | 1,341                              | 1,222           | 119                            | 10%                              |
| <b>Total general and administrative expense</b> | <b>\$ 3,022</b>                    | <b>\$ 3,368</b> | <b>\$ (346)</b>                | <b>(10)%</b>                     |

General and administrative expense for the three months ended December 31, 2018 decreased by \$0.3 million to \$3.0 million compared to the same period in 2017. The decrease was primarily driven by \$0.5 million in lower employee-related costs and share-based based compensation. The decreases were partially offset by \$0.1 million increase in other general and administrative expenses during the three months ended December 31, 2018 compared to the three months ended December 31, 2017.

#### *Comparison of six months ended December 31, 2018 to the six months ended December 31, 2017*

#### *Revenue*

|                              | <b>For the Six Months</b>              |             | <b>Increase<br/>(Decrease)</b> | <b>% Increase<br/>(Decrease)</b> |
|------------------------------|--|-------------|--------------------------------|----------------------------------|
|                              | <b>Ended<br/>December 31,<br/>2018</b> | <b>2017</b> |                                |                                  |
|                              | <b>(dollars in thousands)</b>          |             |                                |                                  |
| Collaboration revenue        |  |             |                                |                                  |
| License and related services | \$ 8,981                               | \$ 13,489   | \$ (4,508)                     | (33)%                            |
| Development services         | 1,961                                  | 1,650       | 311                            | 19%                              |
| Milestone revenue            | 8,978                                  |             | 8,978                          | nm%                              |

|                             |                  |                  |                 |            |
|-----------------------------|------------------|------------------|-----------------|------------|
| Total collaboration revenue | 19,920           | 15,139           | 4,781           | 32%        |
| Grant revenue               | 48               | 28               | 20              | 71%        |
| <b>Total revenue</b>        | <b>\$ 19,968</b> | <b>\$ 15,167</b> | <b>\$ 4,801</b> | <b>32%</b> |

Total revenue for the six months ended December 31, 2018 was \$20.0 million, compared to \$15.2 million for the six months ended December 31, 2017. The increase was primarily due to increased milestone revenue of \$9.0 million and increased development services revenue of \$0.3 million, partially offset by decreased license and related services revenue of \$4.5 million. The increase in milestone revenue was primarily due to recognizing revenue of \$8.3 million associated with the receipt of a \$10.0 million milestone payment from Biogen. Effective July 1, 2018, the Company adopted Topic 606. Based on the Company's Topic 606 revenue recognition methodology, milestone payments are recognized based on the proportional performance of the underlying performance obligation for which the milestone payment relates. The increase in development services revenue was primarily due to additional Phase 1/2 study activities related to the Company's XLRP program. The decrease in license and related service revenue was due to decreased Pre-Funded XLRP activities and due to the Company's revised pattern of revenue recognition under Topic 606. For the six months ended December 31, 2018, license and related services, which includes the Pre-Funded Activities associated with each program under the Biogen Collaboration, are recognized on a proportional performance basis under Topic 606. For the six months ended December 31, 2017, license and related services revenue was recognized based on a straight-line basis over the estimated service period under the Company's prior revenue recognition methodology.

*Research and development expense*

|   | <b>For the Six Months</b>          |             | <b>Increase<br/>(Decrease)</b> | <b>% Increase<br/>(Decrease)</b> |
|---|------------------------------------|-------------|--------------------------------|----------------------------------|
|   | <b>Ended December 31,<br/>2018</b> | <b>2017</b> |                                |                                  |
|   | <b>(dollars in thousands)</b>      |             |                                |                                  |
| <b>External research and development expenses</b> |                                    |             |                                |                                  |
| ACHM  | \$ 1,997                           | \$ 1,754    | \$ 243                         | 14%                              |
| XLRS  | 864                                | 1,338       | (474)                          | (35)%                            |
| XLRP  | 3,095                              | 1,247       | 1,848                          | 148%                             |
| Research and discovery programs                   | 2,035                              | 3,732       | (1,697)                        | (45)%                            |
| <br>  |                                    |             |                                |                                  |
| Total external research and development expenses  | 7,991                              | 8,071       | (80)                           | (1)%                             |
| <b>Internal research and development expenses</b> |                                    |             |                                |                                  |
| Employee-related costs                            | 5,213                              | 4,184       | 1,029                          | 25%                              |
| Share-based compensation                          | 1,025                              | 1,189       | (164)                          | (14)%                            |
| Other   | 3,419                              | 2,558       | 861                            | 34%                              |
| <br>  |                                    |             |                                |                                  |
| Total internal research and development expenses  | 9,657                              | 7,931       | 1,726                          | 22%                              |
| <br>  |                                    |             |                                |                                  |
| Total research and development expense            | \$ 17,648                          | \$ 16,002   | \$ 1,646                       | 10%                              |

Research and development expenses for the six months ended December 31, 2018 were \$17.6 million, compared to \$16.0 million for the six months ended December 31, 2017, an increase of \$1.6 million, or 10%. This increase was primarily attributable to:

\$1.8 million of increased external spending related to XLRP primarily due to incurring sublicense expenses associated with receiving a \$10.0 million XLRP milestone payment from Biogen;

\$1.0 million of increased employee-related expenses associated with the hiring of additional employees to support clinical trial execution and research and development activities; and

\$0.8 million of increased general research and development expenses including training fees, equipment rental fees, dues and conference fees.

These increases were partially offset by:

\$1.7 million of decreased research and discovery spending primarily due to decreased otology and pre-clinical ophthalmology activities; and

\$0.5 million of decreased external XLRS expenses primarily due to reaching full enrollment on the Phase 1/2 clinical trial.

*General and administrative expense*

|   | <b>For the Six<br/>Months</b>      |                 | <b>Increase<br/>(Decrease)</b> | <b>% Increase<br/>(Decrease)</b> |
|---|------------------------------------|-----------------|--------------------------------|----------------------------------|
|   | <b>Ended December 31,<br/>2018</b> | <b>2017</b>     |                                |                                  |
|   | <b>(dollars in thousands)</b>      |                 |                                |                                  |
| Employee-related costs                          | \$ 2,323                           | \$ 2,770        | \$ (447)                       | (16)%                            |
| Share-based compensation                        | 1,250                              | 1,612           | (362)                          | (22)%                            |
| Legal and professional fees                     | 241                                | 337             | (96)                           | (28)%                            |
| Other   | 2,421                              | 2,355           | 66                             | 3%                               |
| <b>Total general and administrative expense</b> | <b>\$ 6,235</b>                    | <b>\$ 7,074</b> | <b>\$ (839)</b>                | <b>(12)%</b>                     |

General and administrative expense for the six months ended December 31, 2018 decreased by \$0.8 million to \$6.2 million compared to the same period in 2017. The decrease was primarily driven by \$0.8 million in lower employee-related costs and share based compensation. Legal and professional fees also decreased by \$0.1 million during the six months ended December 31, 2018 compared to the six months ended December 31, 2017.

## Liquidity and capital resources

We have incurred cumulative losses and negative cash flows from operations since our inception in 1999, and as of December 31, 2018, we had an accumulated deficit of \$136.5 million. It will be several years, if ever, before we have a product candidate ready for commercialization. As noted above, our collaboration agreement with Biogen will terminate effective as of March 8, 2019 and we will not receive any milestone-based or royalty payments under that agreement following its expected termination. Accordingly, we expect that our research and development and general and administrative expenses will continue to increase and as a result, we anticipate that we will require additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

As of December 31, 2018, we had cash, cash equivalents, and investments totaling \$96.1 million. In connection with Bionic Sight's expected IND filing for its optogenetic product in the first half of calendar year 2019, we will be obligated to purchase \$4.0 million of additional equity in Bionic Sight, upon IND and Institutional Review Board clearance. We believe that our existing cash, cash equivalents, and investments at December 31, 2018 will be sufficient to enable us to advance planned preclinical studies and clinical trials for our lead product candidates and currently planned discovery programs for at least the next two years.

Cash in excess of immediate requirements is invested in accordance with our investment policy which primarily seeks to maintain adequate liquidity and preserve capital by generally limiting investments to certificates of deposit and investment-grade debt securities that mature within 24 months. As of December 31, 2018, our cash and cash equivalents were held in bank accounts and money market funds, while our investments consisted of certificates of deposit and corporate and government bonds, none of which mature more than 12 months after the balance sheet date, consistent with our investment policy that seeks to maintain adequate liquidity and preserve capital.

## Cash flows

The following table sets forth the primary sources and uses of cash for each of the periods set forth below:

|  | For the Six Months         |             | Increase<br>(Decrease) | % Increase<br>(Decrease) |
|--|----------------------------|-------------|------------------------|--------------------------|
|  | Ended December 31,<br>2018 | 2017        |                        |                          |
|  | (dollars in thousands)     |             |                        |                          |
| Net cash provided by (used in):                      |                            |             |                        |                          |
| Operating activities                                 | \$ (8,976)                 | \$ (18,500) | \$ 9,524               | 51%                      |
| Investing activities                                 | 1,947                      | 58,154      | (56,207)               | (97)%                    |
| Financing activities                                 | (58)                       | (15)        | (43)                   | 287%                     |
| Net (decrease) increase in cash and cash equivalents | \$ (7,087)                 | \$ 39,639   | \$ (46,726)            | (118)%                   |

**Operating activities.** For the six months ended December 31, 2018 and 2017, net cash used in operating activities was primarily the result of cash received from the collaboration with Biogen, offset by research and development and general and administrative expenses incurred in conducting normal business operation cash payments made for normal business operations and the impact of changes in our working capital accounts.

**Investing activities.** Net cash provided by investing activities for the six months ended December 31, 2018 consisted primarily of cash proceeds of \$46.6 million from the maturity of investments, net of investment repurchases of \$44.5 million. For the six months ended December 31, 2017, net cash provided by investing activities consisted primarily of cash proceeds of \$58.3 million from the maturity of investments.

**Financing activities.** Net cash used in financing activities during the six months ended December 31, 2018 consisted primarily of payments for deferred offering costs, partially offset by proceeds from the exercise of common stock options. Net cash used in financing activities during the six months ended December 31, 2017 was primarily associated with payments toward capital lease obligations, with a minor offset resulting from the exercise of common stock options.

*Operating capital requirements*

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that we will continue to generate losses for the foreseeable future as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products. We are subject to all of the risks incident in the development of new gene therapy products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business.

We believe that our existing cash and cash equivalents and investments at December 31, 2018, will be sufficient to allow us to generate data from our ongoing clinical programs, to move our pre-clinical optogenetic program in collaboration with Bionic Sight into the clinic and to fund our currently planned research and discovery programs for at least the next two years. In order to complete the process of obtaining regulatory approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding.



**ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

Refer to Part II, Item 7A, "Quantitative and Qualitative Disclosures About Market Risk" in our Annual Report on Form 10-K for the year ended June 30, 2018, which is incorporated by reference herein, for a description of our market risks.

**ITEM 4. CONTROLS AND PROCEDURES**

a) Conclusions Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of the Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15e and 15d-15e under the Securities Exchange Act of 1934, as amended as of the end of the period covered by this quarterly report). Based on this evaluation the Chief Executive Officer and Chief Financial Officer concluded that these disclosure controls and procedures are effective and designed to ensure that the information required to be disclosed in our reports filed or submitted under the Exchange Act is recorded, summarized and reported within the requisite time periods.

b) Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15f and 15d-15f under the Exchange Act) identified in connection with the evaluation of our internal control performed during the last fiscal quarter that has materially affected, or is reasonably likely to materially affect our internal control over financial reporting.

## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

We are not a party to any pending legal proceedings. However, because of the nature of our business, we may be subject at any particular time to lawsuits or other claims arising in the ordinary course of our business, and we expect that this will continue to be the case in the future.

### ITEM 1A. RISK FACTORS

Refer to Part I, Item 1A, Risk Factors, in our Annual Report on Form 10-K for the year ended June 30, 2018 for a listing of our risk factors. Material changes to those risk factors since June 30, 2018 are noted below.

***As a result of the expected termination of the Biogen Collaboration Agreement, we will not receive any future milestone-based or royalty payments under the agreement after March 8, 2019.***

On December 7, 2018, Biogen provided us with notice that it was unilaterally terminating the Biogen Collaboration Agreement in its entirety. The expected termination will become effective on March 8, 2019. Under the terms of the Collaboration Agreement, we agreed to collaborate with Biogen to develop, seek regulatory approval for and commercialize gene therapy products to treat X-linked juvenile retinoschisis ( XLRS ) and X-linked retinitis pigmentosa ( XLRP ) based on our adeno-associated virus ( AAV ) vector technologies. The Collaboration Agreement also provided for discovery programs targeting three indications using our AAV technology whereby we would conduct discovery, research and development activities for those additional drug candidates through the stage of clinical candidate designation, after which, Biogen would have been eligible to exercise an option to continue to develop, seek regulatory approval for and commercialize the designated clinical candidate. We also granted Biogen: (i) an exclusive, royalty-bearing license, with the right to grant sublicenses, to use adeno-associated virus vector technology and other technology controlled by us for the purpose of researching, developing, manufacturing and commercializing licensed products developed under the agreement and (ii) a non-exclusive, worldwide, royalty-free, fully paid license, with the right to grant sublicenses, of our interest in other intellectual property developed pursuant to the agreement. Upon the expected termination of the Collaboration Agreement, we will receive back the exclusive license rights to develop, manufacture or commercialize XLRP, XLRS and the three discovery programs. However, as a result of the expected termination of the Collaboration Agreement, we will not receive any future milestone-based or royalty payments under that agreement after March 8, 2019.

***In order to obtain regulatory approval for and commercialize our product candidates, we will need to raise additional funding in the future, which may not be available on acceptable terms, or at all. Failure to obtain necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.***

Other than our product candidates for the treatment of XLRS, XLRP, ACHM CNGB3 and ACHM CNGA3, all of our lead programs in orphan ophthalmology and otology are currently in preclinical development. Developing gene therapy products is expensive, and we expect our research and development expenses to increase substantially as we advance our current product candidates in clinical trials and as we undertake preclinical studies of new product candidates.

Our operations have consumed substantial amounts of cash since inception. As of December 31, 2018, and 2017, our cash and cash equivalents and investments amounted to \$96.1 million and \$119.7 million, respectively. Our research and development expenses were \$32.2 million, \$26.2 million and \$39.4 million for the fiscal years ended June 30,

2018, 2017 and 2016, respectively. We believe that our existing cash and cash equivalents at December 31, 2018 will be sufficient to enable us to advance planned preclinical studies and clinical trials for our lead product candidates for at least the next two years. In order to complete the process of obtaining regulatory approval for our lead product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our lead product candidates, if approved, we will require substantial additional funding. Also, our current operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches.

Any such fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, financing may not be available to us in the future in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and a portion of our operating cash flows, if any, being dedicated to the payment of principal and interest on such indebtedness, and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and we may be required to relinquish or license on unfavorable terms rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, financial condition, results of operations and prospects and cause the price of our common stock to decline.

As a result of the expected termination of the Biogen Collaboration Agreement, we will not receive any future milestone-based or royalty payments under that agreement after March 8, 2019 which will make it more likely that we will need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches. If we are unable to obtain needed funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition, results of operations, cash flows and prospects and cause the price of our common stock to decline.

***Collaborations with third parties may be important to our business. If these collaborations are not successful, our business could be adversely affected.***

In addition to our current collaborations with Biogen, Synpromics, the University of Florida and Bionic Sight, we may in the future seek third-party collaborators for the development and commercialization of product candidates based on our gene therapy platform. If we enter into such collaborations, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from any future collaboration or license agreement will depend on the collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any collaborators may have the right to abandon research or development projects and terminate applicable agreements, including any funding obligations, prior to or upon the expiration of the agreed upon terms. For example, on December 7, 2018, we received notice from Biogen that our collaboration agreement with them would be terminated effective March 8, 2019. As a result of the expected termination, we will not receive any future milestone-based or royalty payments under the Collaboration Agreement after March 8, 2019.

Our current collaborations or any collaboration we enter into in the future, may also pose a number of risks, including the following:

collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;

collaborators may not perform their obligations as expected;

exclusivity rights we negotiate with our collaborators may be unenforceable in certain jurisdictions;

collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;

collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

collaborators may decide not to continue the development of collaboration products and could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;

take-over or step-in rights granted to a collaborator with respect to one or more of our product candidates, may cause us to have limited control over future development activities and/or realize diminished economic or other benefits upon the ultimate commercialization of that product candidate;

a collaborator with marketing, distribution and commercialization rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;

if we fail to obtain orphan product designation for a partnered product, we may realize diminished economic benefit upon the ultimate commercialization of that product candidate;

restrictions and commitments contained in collaborations may have the effect of preventing us from independently undertaking development and other efforts that may appear to be attractive to us;

disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development of any product candidates, might cause delays or termination of the research, development or commercialization of such product candidates, might lead to additional responsibilities for us with respect to such product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;

collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;

collaborations may be terminated at the convenience of the collaborator or for a material breach by either party, and, if a collaboration is terminated, we could be required to make payments to the collaborator or have our potential payments under the collaboration reduced; and

in the event of the termination of a collaboration, like the expected termination of the Biogen Collaboration Agreement effective as of March 8, 2019, we could be required to raise additional capital to pursue further development or commercialization of the product candidates returned to us by our former collaborator.

If our collaborations do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our

development of our gene therapy platform and product candidates could be delayed and we may need additional resources to develop product candidates and gene therapy platform. As a result of these or other factors, we may not receive the benefits that we expect from our collaborations.

Additionally, subject to its contractual obligations to us, if one of our collaborators is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

We may in the future determine to collaborate with other pharmaceutical and biotechnology companies for development and potential commercialization of product candidates. These relationships or those like them may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement with any such new party will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If we license product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenues or specific net income that justifies such transaction.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our gene therapy platform and our business may be materially and adversely affected.

## ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

The following table provides certain information with respect to our purchases of shares of the Company's common stock during the second fiscal quarter of 2019:

### Issuer Purchases of Equity Securities

| Period                                     | Total Number of Shares Purchased <sup>(a)</sup> | Average Price Paid per Share | Total Number of Shares Purchased as Part of Publicly Announced Plan | Approximate Dollar Value of Shares That May Yet Be Purchased Under the Plan |
|--|---|------------------------------|---|---|
| October 1, 2018 through October 31, 2018   | 612   | \$ 6.46                      |   | \$  |
| November 1, 2018 through November 30, 2018 | 685   | \$ 7.05                      |   | \$  |
| December 1, 2018 through December 31, 2018 | 611   | \$ 6.11                      |   | \$  |
| Total                                      | 1,908   | \$ 6.56                      |   |   |

- (a) These columns reflect the surrender to the Company of an aggregate of 1,908 shares of common stock to satisfy tax withholding obligations in connection with the vesting of restricted stock issued to an employee during the second fiscal quarter of 2019.

## ITEM 6. EXHIBITS

| Exhibit | Description |
|---------|-------------|
|---------|-------------|



**Number**

- 3.1 Fifth Amended and Restated Certificate of Incorporation of Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, event date March 26, 2014, filed on April 1, 2014)
- 3.2 Amended and Restated Bylaws of Applied Genetic Technologies Corporation (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, event date March 26, 2014, filed on April 1, 2014)
- 31.1\* Rule 13a-14(a)/15d-14(a) Certification of Principal Executive Officer of Applied Genetic Technologies Corporation
- 31.2\* Rule 13a-14(a)/15d-14(a) Certification of Principal Financial Officer of Applied Genetic Technologies Corporation
- 32.1\*\* Section 1350 Certification of Principal Executive Officer and Principal Financial Officer of Applied Genetic Technologies Corporation
- 101\* Interactive Data Files pursuant to Rule 405 of Regulation S-T (XBRL)

\* Filed herewith.

\*\* Furnished herewith.

**SIGNATURE**

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

APPLIED GENETIC TECHNOLOGIES  
CORPORATION

(Registrant)

By: /s/ William Sullivan  
William Sullivan, Chief Financial Officer

Date: February 7, 2019