AGIOS PHARMACEUTICALS INC
Form 10-Q
August 08, 2017
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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 June 30, 2017
OR
TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
Commission file number 001-36014

AGIOS PHARMACEUTICALS, INC.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	26-0662915
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)

88 Sidney Street, Cambridge, Massachusetts	02139
(Address of Principal Executive Offices)	(Zip Code)

(617) 649-8600
(Registrant's Telephone Number, Including Area Code)
(Former Name, Former Address and Former Fiscal Year, if Changed Since Last Report)

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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes No
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer	Accelerated filer
Non-accelerated filer (Do not check if a smaller reporting company)	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. Yes No
Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

Number of shares of the registrant's Common Stock, $0.001 par value, outstanding on August 4, 2017: 48,391,194

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements (Unaudited)

AGIOS PHARMACEUTICALS, INC.

Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)

(Unaudited)

	June 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$138,717	$160,754
Marketable securities	447,325	380,560
Collaboration receivable – related party	4,842	4,886
Tenant improvement and other receivables	779	3,428
Prepaid expenses and other current assets	13,018	10,264
Total current assets	604,681	559,892
Marketable securities	129,899	32,250
Property and equipment, net	24,771	25,337
Other non-current assets	1,249	1,615
Total assets	$760,600	$619,094
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$23,409	$17,106
Accrued expenses	29,641	32,002
Deferred revenue – related party	37,069	35,913
Deferred rent	3,529	3,412
Total current liabilities	93,648	88,433
Deferred revenue, net of current portion – related party	141,957	154,297
Deferred rent, net of current portion	16,003	17,773
Total liabilities	251,608	260,503
Stockholders' equity:		
Preferred stock, $0.001 par value; 25,000,000 shares authorized; no shares issued or outstanding at June 30, 2017 and December 31, 2016	—	—
Common stock, $0.001 par value; 125,000,000 shares authorized; 48,346,297 and 42,220,444 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	48	42
Additional paid-in capital	1,142,233	842,013
Accumulated other comprehensive loss	(650)	(313)
Accumulated deficit	(632,639)	(483,151)
Total stockholders' equity	508,992	358,591
Total liabilities and stockholders' equity	$760,600	$619,094

See accompanying Notes to Condensed Consolidated Financial Statements.

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AGIOS PHARMACEUTICALS, INC.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Collaboration revenue – related party	$11,346	$6,978	$21,854	$38,259
Operating expenses:				
Research and development (net of $2,489 and $5,922 of cost reimbursement from related party for the three months ended June 30, 2017 and 2016, respectively, and $5,265 and $14,716 of cost reimbursement from related party for the six months ended June 30, 2017 and 2016, respectively)	79,816	50,804	142,548	94,842
General and administrative	16,130	12,644	30,953	23,481
Total operating expenses	95,946	63,448	173,501	118,323
Loss from operations	(84,600)	(56,470)	(151,647)	(80,064)
Interest income	1,518	517	2,399	913
Net loss	$(83,082)	$(55,953)	$(149,248)	$(79,151)
Net loss per share – basic and diluted	$(1.78)	$(1.47)	$(3.35)	$(2.09)
Weighted-average number of common shares used in computing net loss per share – basic and diluted	46,745,760	37,956,383	44,525,478	37,910,233

See accompanying Notes to Condensed Consolidated Financial Statements.

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AGIOS PHARMACEUTICALS, INC.
Condensed Consolidated Statements of Comprehensive Loss
(in thousands)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net loss	$(83,082)	$(55,953)	$(149,248)	$(79,151)
Other comprehensive (loss) income				
Unrealized (loss) gain on available-for-sale securities	(438)	208	(337)	615
Comprehensive loss	$(83,520)	$(55,745)	$(149,585)	$(78,536)

See accompanying Notes to Condensed Consolidated Financial Statements.

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AGIOS PHARMACEUTICALS, INC.
Condensed Consolidated Statements of Cash Flows
(in thousands)
(Unaudited)

	Six Months Ended June 30,	
	2017	2016
Operating activities		
Net loss	$(149,248)	$(79,151)
Adjustments to reconcile net loss cash (used in) provided by operating activities:		
Depreciation	3,164	2,550
Stock-based compensation expense	22,921	20,103
Net amortization of premium and discounts on investments	94	330
Loss on disposal of property and equipment	40	—
Changes in operating assets and liabilities:		
Collaboration receivable – related party	44	372
Tenant improvement and other receivables	2,638	1,360
Prepaid expenses and other current and non-current assets	(2,417)	(1,129)
Accounts payable	4,930	3,755
Accrued expenses	(2,392)	(833)
Deferred revenue – related party	(11,184)	190,645
Deferred rent	(1,653)	813
Net cash (used in) provided by operating activities	(133,063)	138,815
Investing activities		
Purchases of marketable securities	(468,556)	(226,289)
Proceeds from maturities and sales of marketable securities	303,711	252,468
Purchases of property and equipment	(1,328)	(5,267)
Net cash (used in) provided by investing activities	(166,173)	20,912
Financing activities		
Reimbursement of public offering costs, net of payments	104	—
Proceeds from public offering of common stock, net of commissions	270,250	—
Net proceeds from stock option exercises and employee stock purchase plan	6,845	2,557
Net cash provided by financing activities	277,199	2,557
Net change in cash and cash equivalents	(22,037)	162,284
Cash and cash equivalents at beginning of the period	160,754	71,764
Cash and cash equivalents at end of the period	$138,717	$234,048
Supplemental disclosure of non-cash investing and financing transactions		
Additions to property and equipment in accounts payable and accrued expenses	$1,383	$1,360
Proceeds from stock option exercises in other receivables	$3	$23
Public offering costs in other receivables, net of amounts in accounts payable and accrued expenses	$125	$—

See accompanying Notes to Condensed Consolidated Financial Statements.

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AGIOS PHARMACEUTICALS, INC.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

1. Overview and Basis of Presentation

References to Agios

Throughout this Quarterly Report on Form 10-Q, "we," "us," and "our," and similar expressions, except where the context requires otherwise, refer to Agios Pharmaceuticals, Inc. and its consolidated subsidiaries, and "our Board of Directors" refers to the board of directors of Agios Pharmaceuticals, Inc.

Overview

We are a biopharmaceutical company committed to the fundamental transformation of patients' lives through scientific leadership in the field of cellular metabolism, with the goal of making transformative, first- or best-in-class medicines. Our areas of focus are cancer metabolism, rare genetic diseases, or RGDs, which are diseases that are directly caused by changes in genes or chromosomes, often passed from one generation to the next, and metabolic immuno-oncology, or MIO, which is a developing field that aims to modulate the activity of relevant immune cells (or tumor microenvironment) by targeting critical metabolic nodes, thereby enhancing the immune mediated anti-tumor response. In each of these areas, we are seeking to unlock the biology of cellular metabolism as a platform to create transformative therapies. We are located in Cambridge, Massachusetts.

Basis of presentation

The condensed consolidated balance sheet as of June 30, 2017, and the condensed consolidated statements of operations and comprehensive loss for the three and six months ended June 30, 2017 and 2016, and cash flows for the six months ended June 30, 2017 and 2016, are unaudited. The unaudited condensed consolidated financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of our management, reflect all adjustments, which include only normal recurring adjustments, necessary to fairly state our financial position as of June 30, 2017, and our results of operations for the three and six months ended June 30, 2017 and 2016, and cash flows for the six months ended June 30, 2017 and 2016. The financial data and the other financial information disclosed in these notes to the condensed consolidated financial statements related to the three and six-month period are also unaudited. The results of operations for the three and six months ended June 30, 2017 are not necessarily indicative of the results to be expected for the year ending December 31, 2017 or for any other future annual or interim period. The year-end condensed consolidated balance sheet data was derived from our audited financial statements, but does not include all disclosures required by U.S. generally accepted accounting principles, or U.S. GAAP. Accordingly, the condensed consolidated interim financial statements should be read in conjunction with the audited consolidated financial statements and notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2016 that was filed with the Securities and Exchange Commission, or the SEC, on February 16, 2017.

Our consolidated financial statements include our accounts and the accounts of our wholly owned subsidiaries, Agios Securities Corporation and Agios International Sarl. All intercompany transactions have been eliminated in consolidation. The consolidated financial statements have been prepared in conformity with U.S. GAAP.

Liquidity

In April 2017, we completed a public offering of 5,050,505 shares of common stock at an offering price of $49.50 per share. We received net proceeds from this offering of $235.0 million, after deducting underwriting discounts and commissions paid by us. In addition, we granted the underwriters the right to purchase up to an additional 757,575 shares of common stock, which was also exercised in April 2017, resulting in additional net proceeds to us of $35.2 million, after underwriting discounts and commissions. After giving effect to the full exercise of the over-allotment option, the total number of shares sold by us in the public offering increased to 5,808,080 shares, and net proceeds to us increased to $270.2 million, after underwriting discounts and commissions.

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2. Summary of Significant Accounting Policies and Recent Accounting Pronouncements

Significant accounting policies

In the quarter ended March 31, 2017, we adopted Accounting Standards Update, or ASU, 2016-09, Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting, or ASU 2016-09, which simplifies several aspects of the accounting for employee share-based payment transactions. There have been no other material changes to the significant accounting policies previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2016.

Recent accounting pronouncements

Revenue from Contracts with Customers

In May 2014, the Financial Accounting Standards Board, or FASB, issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), or ASU 2014-09. Subsequently, the FASB issued ASU 2015-14, Revenue from Contracts with Customers (Topic 606), which adjusted the effective date of ASU 2014-09; ASU No. 2016-08, Revenue from Contracts with Customers (Topic 606): Principal versus Agent Considerations (Reporting Revenue Gross versus Net), which amends the principal-versus-agent implementation guidance and illustrations in ASU 2014-09; ASU No. 2016-10, Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing, which clarifies identifying performance obligations and licensing implementation guidance and illustrations in ASU 2014-09; and ASU No. 2016-12, Revenue from Contracts with Customers (Topic 606): Narrow-Scope Improvements and Practical Expedients, which addresses implementation issues and is intended to reduce the cost and complexity of applying the new revenue standard in ASU 2014-09, or collectively, the Revenue ASUs.

The Revenue ASUs provide an accounting standard for a single comprehensive model for use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance. The accounting standard is effective for interim and annual periods beginning after December 15, 2017, with an option to early adopt for interim and annual periods beginning after December 15, 2016. The guidance permits two methods of adoption: retrospectively to each prior reporting period presented (the full retrospective method), or retrospectively with the cumulative effect of initially applying the guidance recognized at the date of initial application (the modified retrospective method). We will adopt the new standard effective January 1, 2018 under the modified retrospective method. We have allocated internal resources to the implementation and are in the process of determining the impact of the Revenue ASUs on our financial statements; however, the adoption of the Revenue ASUs may have a material impact on revenue recognition, our notes to consolidated financial statements and our internal controls over financial reporting. As discussed further in detail within Note 5, Collaboration Agreements, our collaboration agreements with Celgene Corporation, or Celgene, is currently our sole source of revenue and the only arrangement impacted by the adoption of the Revenue ASUs.

Other Recent Accounting Pronouncements

In May 2017, the FASB issued ASU No. 2017-09, Compensation - Stock Compensation (Topic 718): Scope of Modification Accounting, or ASU 2017-09, which provides guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Accounting Standards Codification, or ASC, 718. ASU 2017-09 is effective for us for annual periods beginning after December 15, 2017, and interim periods therein, with early adoption permitted. We are currently in the process of evaluating the impact of the guidance on our consolidated financial statements.

In October 2016, the FASB issued ASU No. 2016-16, Income Taxes (Topic 740): Intra-Entity Transfers of Assets Other Than Inventory, or ASU 2016-16, which removes the prohibition in ASC 740, Income Taxes, against the immediate recognition of the current and deferred income tax effects of intra-entity transfers of assets other than inventory. ASU 2016-16 is effective for us for annual periods beginning after December 15, 2017, and interim periods therein, with early adoption permitted. We are currently in the process of evaluating the impact of the guidance on our consolidated financial statements.

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842), or ASU 2016-02, which establishes principles that lessees and lessors shall apply to report useful information to users of financial statements about the

amount, timing and uncertainty of cash flows arising from a lease. ASU 2016-02 is effective for us for annual periods beginning after December 15, 2018 and interim periods therein, with early adoption permitted. We are currently in the process of evaluating the impact of the guidance on our consolidated financial statements.

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Other accounting standards that have been issued by the FASB or other standards-setting bodies that do not require adoption until a future date are not expected to have a material impact on our financial statements upon adoption.

3. Fair Value Measurements

We record cash equivalents and marketable securities at fair value. ASC 820, Fair Value Measurements and Disclosures, establishes a fair value hierarchy for those instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and our own assumptions (unobservable inputs). The hierarchy consists of three levels:

Level 1 – Unadjusted quoted prices in active markets for identical assets or liabilities.

Level 2 – Quoted prices for similar assets and liabilities in active markets, quoted prices in markets that are not active, or inputs which are observable, directly or indirectly, for substantially the full term of the asset or liability.

Level 3 – Unobservable inputs that reflect our own assumptions about the assumptions market participants would use in pricing the asset or liability in which there is little, if any, market activity for the asset or liability at the measurement date.

The following table summarizes the cash equivalents and marketable securities measured at fair value on a recurring basis as of June 30, 2017 (in thousands):

	Level 1	Level 2	Level 3	Total
Cash equivalents	$91,766	$23,506	$ —	$115,272
Marketable securities:				
Certificates of deposit	—	14,060	—	14,060
U.S. Treasuries	156,452	—	—	156,452
Government securities	43,185	59,658	—	102,843
Corporate debt securities	—	303,869	—	303,869
Total cash equivalents and marketable securities	$291,403	$401,093	$ —	$692,496

Cash equivalents and marketable securities have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, utilizing third-party pricing services or other market observable data. The pricing services utilize industry standard valuation models, including both income and market based approaches, and observable market inputs to determine value. After completing our validation procedures, we did not adjust or override any fair value measurements provided by the pricing services as of June 30, 2017.

There have been no changes to the valuation methods during the six months ended June 30, 2017. We evaluate transfers between levels at the end of each reporting period. Due to the lack of an active market, there were $1.0 million in transfers of assets or liabilities between Level 1 and Level 2 during the six months ended June 30, 2017. We have no financial assets or liabilities that were classified as Level 3 at any point during the six months ended June 30, 2017.

4. Marketable Securities

Marketable securities at June 30, 2017 and December 31, 2016 consisted of investments in certificates of deposit, U.S. Treasuries, government securities and corporate debt securities. We determine the appropriate classification of the securities at the time they are acquired and evaluate the appropriateness of such classifications at each balance sheet date. We classify our marketable securities as available-for-sale pursuant to ASC 320, Investments – Debt and Equity Securities. Marketable securities are recorded at fair value, with unrealized gains and losses included as a component of accumulated other comprehensive loss in stockholders' equity and a component of total comprehensive loss in the condensed consolidated statements of comprehensive loss, until realized. Realized gains and losses are included in investment income on a specific-identification basis. There were no realized gains or losses on marketable securities for the three and six months ended June 30, 2017 and 2016 and, as a result, there were no reclassifications of any amounts out of accumulated other comprehensive loss for those periods.

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Marketable securities at June 30, 2017 consist of the following (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Current:				
Certificates of deposit	$ 12,400	$ 1	$ (12)	$12,389
U.S Treasuries	118,262	1	(105)	118,158
Government securities	84,931	—	(61)	84,870
Corporate debt securities	232,029	18	(139)	231,908
Non-current:				
Certificates of deposit	1,680	—	(9)	1,671
U.S Treasuries	38,405	—	(111)	38,294
Government securities	18,011	—	(38)	17,973
Corporate debt securities	72,156	1	(196)	71,961
	$ 577,874	$ 21	$ (671)	$577,224

Marketable securities at December 31, 2016 consist of the following (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Current:				
Certificates of deposit	$ 11,280	$ 2	$ (3)	$11,279
U.S. Treasuries	141,678	2	(62)	141,618
Government securities	19,533	—	(23)	19,510
Corporate debt securities	208,285	3	(135)	208,153
Non-current:				
Certificates of deposit	7,600	6	(13)	7,593
Government securities	4,499	—	(21)	4,478
Corporate debt securities	20,248	—	(69)	20,179
	$ 413,123	$ 13	$ (326)	$412,810

At June 30, 2017 and December 31, 2016, we held both current and non-current investments. Investments classified as current have maturities of less than one year. Investments classified as non-current are those that: (i) have a maturity of one to two years, and (ii) we do not intend to liquidate within the next twelve months, although these funds are available for use and therefore classified as available-for-sale.

We review marketable securities for other-than-temporary impairment whenever the fair value of a marketable security is less than the amortized cost and evidence indicates that a marketable security's carrying amount is not recoverable within a reasonable period of time. Other-than-temporary impairments of investments are recognized in the condensed consolidated statements of operations if we experienced a credit loss, have the intent to sell the marketable security, or if it is more likely than not that we will be required to sell the marketable security before recovery of the amortized cost basis. Evidence considered in this assessment includes reasons for the impairment, compliance with our investment policy, the severity and the duration of the impairment, and changes in value subsequent to the end of the period.

At June 30, 2017 and December 31, 2016, we held 255 and 158 debt securities that were in an unrealized loss position for less than one year, respectively. The aggregate fair value of debt securities in an unrealized loss position at June 30, 2017 and December 31, 2016 was $482.7 million and $335.4 million, respectively. There were no individual securities that were in a significant unrealized loss position as of June 30, 2017 and December 31, 2016. We evaluated our securities for other-than-temporary impairment and considered the decline in market value for the securities to be primarily attributable to current economic and market conditions. It is not more likely than not that we will be required to sell the securities, and we do not intend to do so prior to the recovery of the amortized cost basis. Based on this analysis, these marketable securities were not considered to be other-than-temporarily impaired as of June 30, 2017 and December 31, 2016.

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5. Collaboration Agreements

Celgene Corporation

To date, our revenue has primarily been generated from our collaboration agreements with Celgene, or collectively, the Collaboration Agreements. Celgene is a related party through ownership of our common stock. In April 2010, we entered into a collaboration agreement focused on cancer metabolism. The agreement was amended in October 2011 and July 2014, or collectively the agreement together with the amendments, the 2010 Agreement. On April 27, 2015, we entered into a joint worldwide development and profit share collaboration and license agreement with Celgene, and our wholly owned subsidiary, Agios International Sarl, entered into a collaboration and license agreement with Celgene International II Sarl, or collectively, the AG-881 Agreements, to establish a worldwide collaboration focused on the development and commercialization of AG-881 products. On May 17, 2016, we entered into a master research and collaboration agreement, or the 2016 Agreement.

2016 Agreement

In May 2016, we entered into the 2016 Agreement focused on MIO. In addition to new programs identified under the 2016 Agreement, both parties also agreed that all future development and commercialization of two remaining cancer metabolism programs discovered under the 2010 Agreement, including AG-270, a program focused on methylthioadenosine phosphorylase, or MTAP, deleted cancers, will now be governed by the 2016 Agreement.

During the research term of the 2016 Agreement, we plan to conduct research programs focused on discovering compounds that are active against metabolic targets in the immuno-oncology, or IO, field. The initial four-year research term will expire on May 17, 2020, and may be extended for up to two, or in specified cases, up to four additional one-year terms.

For each program under the 2016 Agreement, we may nominate compounds that meet specified criteria as development candidates and, in limited circumstances, Celgene may also nominate compounds as development candidates for each such program. Celgene may designate the applicable program for further development following any such nomination, after which we may conduct, at our expense, additional preclinical and clinical development for such program through the completion of an initial phase 1 dose escalation study.

At the end of the research term, Celgene may designate for continued development up to three research programs for which development candidates have yet to be nominated, which are referred to as continuation programs. We may conduct further research and preclinical and clinical development activities on any continuation program, at our expense, through the completion of an initial phase 1 dose escalation study.

We granted Celgene the right to obtain exclusive options for development and commercialization rights for each program that Celgene has designated for further development, and for each continuation program. Celgene may exercise each such option beginning on the designation of a development candidate for such program (or on the designation of such program as a continuation program) and ending on the earlier of: (i) the end of a specified period after we have furnished Celgene with specified information about the initial phase 1 dose escalation study for such program, or (ii) January 1, 2030. Research programs that have applications in the inflammation or autoimmune, or I&I, field that may result from the 2016 Agreement will also be subject to the exclusive options described above. We will retain rights to any program that Celgene does not designate for further development or as to which it does not exercise its option.

Under the terms of the 2016 Agreement, following Celgene's exercise of its option with respect to a program, the parties will enter into either a co-development and co-commercialization agreement if such program is in the IO field, or a license agreement if such program is in the I&I field. Under each co-development and co-commercialization agreement, the two parties will co-develop and co-commercialize licensed products worldwide. Either we or Celgene will lead development and commercialization of licensed products for the United States, and Celgene will lead development and commercialization of licensed products outside of the United States. Depending on the country, the parties will each have the right to provide a portion of field-based marketing activities. Under each license agreement, Celgene will have the sole right to develop and commercialize licensed products worldwide.

Co-development and co-commercialization agreements

Under each co-development and co-commercialization agreement entered into under the 2016 Agreement, the parties will split all post-option exercise worldwide development costs, subject to specified exceptions, as well as any profits

from any net sales

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of, or commercialization losses related to, licensed products in the IO field. Celgene has the option to designate one program in the IO field as the 65/35 program, for which Celgene will be the lead party for the United States and will have a 65% profit or loss share. For programs in the IO field other than the 65/35 program, we and Celgene will alternate, on a program-by-program basis, being the lead party for the United States, with us having the right to be the lead party for the first such program, and each party will have a 50% profit or loss share. The lead party for the United States will book commercial sales of licensed products, if any, in the United States, and Celgene will book commercial sales of licensed products, if any, outside of the United States.

License agreements

Under each license agreement under the 2016 Agreement, Celgene will be responsible for all post-option exercise worldwide development and associated costs, subject to specified exceptions, as well as worldwide commercialization and associated costs, for licensed products.

Financial terms

Under the terms of the 2016 Agreement, we received an initial upfront payment in the amount of $200.0 million. The 2016 Agreement provides specified rights to extend the research term for up to two, or in specified cases, up to four, additional years by paying a $40.0 million per-year extension fee. Celgene will pay an $8.0 million designation fee for each program that Celgene designates for further development and for each continuation program. During the three months ended March 31, 2017, we received $8.0 million from Celgene upon the designation of AG-270, our program focused on MTAP-deleted cancers, as a development candidate. For each program as to which Celgene exercises its option to develop and commercialize, subject to antitrust clearance, Celgene will pay an option exercise fee of at least $30.0 million for any designated development program and at least $35.0 million for any continuation programs. In certain cases, Celgene may exercise its option to develop and commercialize two early-stage I&I programs, prior to Celgene designating the program for further development, by paying an option exercise fee of $10.0 million.

We are eligible to receive the following milestone-based payments associated with the 2016 Agreement:

Program	Milestone	Amount
65/35 program in IO field	Specified clinical development event	$25.0 million
65/35 program in IO field	Specified regulatory milestone events	Up to $183.8 million
50/50 program in IO field	Specified clinical development event	$20.0 million
50/50 program in IO field	Specified regulatory milestone events	Up to $148.8 million
I&I field	Specified clinical development event	$25.0 million
I&I field	Specified regulatory milestone events	Up to $236.3 million
I&I field	Specified commercial milestone events	Up to $125.0 million

Additionally, for each licensed program in the I&I field, we are eligible to receive royalties at tiered, low double-digit percentage rates on Celgene’s net sales, if any.

Opt-out right

Under the 2016 Agreement, we may elect to opt out of the cost and profit share under any co-development and co-commercialization agreement, subject to specified exceptions. Upon opting out, Celgene will have the sole right to develop, manufacture and commercialize the applicable licensed products throughout the world, at its cost, and we will undertake transitional activities reasonably necessary to transfer the development, manufacture and commercialization of such licensed products to Celgene, at our expense. Further, in lieu of the profit or loss sharing described above, we would be eligible to receive royalties at tiered, low double-digit percentage rates on Celgene’s net sales, if any, of the applicable licensed products. However, we would continue to be eligible to receive the developmental and regulatory milestone-based payments described above.

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Term

The term of the 2016 Agreement commenced on May 17, 2016 and, if not terminated earlier, will expire upon the later of the last-to-expire of the research term and all option exercise periods, or, if an option is exercised by Celgene for one or more programs in the collaboration, upon the termination or expiration of the last-to-exist co-development and co-commercialization agreement or license agreement, as applicable, for any such program.

Termination

Subject to specified exceptions, Celgene may terminate the 2016 Agreement in its entirety for any reason by providing us with prior written notice if there are no active co-development and co-commercialization agreements or license agreements in place or on a program-by-program basis if there are no active co-development and co-commercialization agreements or license agreements in place for the terminated program(s). Either party may terminate the 2016 Agreement for the insolvency of the other party. On a program-by-program basis, prior to the exercise of an option, either party may terminate the 2016 Agreement either in its entirety or with respect to one or more programs on prior written notice to the other party in the case of an uncured material breach by the other party that frustrates the fundamental purpose of the 2016 Agreement. Following the exercise of an option for a program, either party may terminate the 2016 Agreement with respect to such program if such party terminates the co-development and co-commercialization agreement or license agreement for such program for an uncured material breach by the other party that frustrates the fundamental purpose of such agreement. Either party may terminate a co-development and co-commercialization agreement or a license agreement upon the bankruptcy or insolvency of the other party. Either party also has the right to terminate the co-development and co-commercialization agreement or license agreement if the other party or any of their affiliates challenges the validity, scope or enforceability of or otherwise opposes, any patent included within the intellectual property rights licensed to the other party under such agreement.

Exclusivity

While any of Celgene's options remain available under the 2016 Agreement, subject to specified exceptions, we may not directly or indirectly develop, manufacture or commercialize, outside of the 2016 Agreement, any therapeutic modality in the IO or I&I field with specified activity against a metabolic target.

During the term of each co-development and co-commercialization agreement and license agreement, subject to specified exceptions, neither we nor Celgene may directly or indirectly develop, manufacture or commercialize outside of such agreement any therapeutic modality in any field with specified activity against the metabolic target that is the focus of the program licensed under such agreement.

AG-120 Letter Agreement

On May 17, 2016, we entered into a letter agreement with Celgene regarding ivosidenib, or the AG-120 Letter Agreement. Under the AG-120 Letter Agreement, the parties agreed to terminate the 2010 Agreement, effective as of August 15, 2016, as to the program directed to the isocitrate dehydrogenase 1, or IDH1, target, for which ivosidenib is the lead development candidate. Under the 2010 Agreement, Celgene had held development and commercialization rights to the IDH1 program outside of the United States, and we held such rights inside the United States. As a result of the termination, we obtained global rights to ivosidenib and the IDH1 program. Neither party will have any further financial obligation, including royalties or milestone payments, to the other concerning ivosidenib or the IDH1 program. Under the terms of the termination, the parties have also agreed to conduct specified transitional activities in connection with the termination. In addition, pursuant to the AG-120 Letter Agreement, the parties are released from their exclusivity obligations under the 2010 Agreement with respect to the IDH1 program. The termination does not affect the AG-881 Agreements, which are directed to both the IDH1 target and the isocitrate dehydrogenase 2, or IDH2, target.

AG-881 Agreements

On April 27, 2015, we entered into the AG-881 Agreements. The AG-881 Agreements establish a joint worldwide collaboration focused on the development and commercialization of AG-881 products. Under the terms of the AG-881 Agreements, we received an initial upfront payment of $10.0 million in May 2015 and are eligible to receive milestone-based payments described below. The parties will split all worldwide development costs equally, subject to specified exceptions, as well as any profits from any net sales of, or commercialization losses related to, licensed

AG-881 products.

We are eligible to receive up to $70.0 million in potential milestone payments under the AG-881 Agreements. The potential milestone payments are comprised of: (i) a $15.0 million milestone payment for filing of a first new drug application, or NDA,

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in a major market, and (ii) up to $55.0 million in milestone payments upon achievement of specified regulatory milestone events. We may also receive royalties at tiered, low-double digit to mid-teen percentage rates on net sales if we elect not to participate in the development and commercialization of AG-881.

2010 Agreement

In April 2010, we entered into the 2010 Agreement, which was amended in October 2011 and July 2014. The goal of the collaboration was to discover, develop and commercialize disease-altering therapies in oncology based on our cancer metabolism research platform. We initially led discovery, preclinical and early clinical development for all cancer metabolism programs under the collaboration. The discovery phase of the 2010 Agreement expired in April 2016.

Upon agreement to terminate the 2010 Agreement, effective as of August 15, 2016, as to the program directed to the IDH1 target, for which ivosidenib is the lead development candidate, the sole program remaining under the 2010 Agreement is IDHIFA®, a co-commercialized licensed program for which Celgene leads and funds global development and commercialization activities. We have exercised our right to participate in a portion of commercialization activities in the United States for IDHIFA® in accordance with the applicable commercialization plan. On August 1, 2017, the FDA granted Celgene approval of IDHIFA® for the treatment of adult patients with relapsed or refractory acute myeloid leukemia, or R/R AML, with an IDH2 mutation as detected by an FDA-approved test.

Under the remaining terms of the 2010 Agreement, we are eligible to receive up to $95.0 million in potential milestone payments for the IDHIFA® program. The potential milestone payments are comprised of: (i) up to $70.0 million in milestone payments upon achievement of specified ex-U.S. regulatory milestone events, and (ii) a $25.0 million milestone payment upon achievement of a specified commercial milestone event.

Under the 2010 Agreement, we may also receive royalties at tiered, low-double digit to mid-teen percentage rates on net sales and have the option to participate in the development and commercialization of certain products in the United States. Assuming all other revenue recognition criteria are met, royalty payments will be recognized as revenue in the period in which they are earned. To date, we have not earned any royalty payments under the 2010 Agreement.

Unless terminated earlier by either party, the term of the 2010 Agreement will continue until the expiration of all royalty terms with respect to IDHIFA®. Celgene may terminate this agreement for convenience in its entirety upon ninety days written notice to us. If either party is in material breach and fails to cure such breach within the specified cure period, the other party may terminate the 2010 Agreement in its entirety. Either party may terminate the agreement in the event of specified insolvency events involving the other party.

Accounting analysis and revenue recognition – collaboration revenue

April 2015 modification: The AG-881 Agreements were determined to be a modification of the 2010 Agreement because they govern AG-881, a compound originally identified within the 2010 Agreement.

May 2016 modification: The 2016 Agreement was determined to be a modification of the 2010 Agreement and the AG-881 Agreements because it governs compounds originally identified within the 2010 Agreement, including AG-270. All undelivered elements identified under the April 2015 modification that remained undelivered at the time of the May 2016 modification are supplanted by the undelivered elements identified under the May 2016 modification.

Upon the modifications, under the provisions of ASC 605-25, Multiple Element Arrangements, we identified the remaining deliverables under the modified arrangement, and determined the best estimate of selling price, or BESP, for the undelivered elements as of the modification date. We then allocated the total arrangement consideration, which included the remaining deferred revenue balance at the modification date, any upfront payments received upon modification and other consideration under the modified arrangement that were deemed to be determinable at the modification date, to each unit of accounting relative to our BESP for each unit of accounting. The undelivered elements at the time of the modification, which are each considered by us to have stand-alone value and therefore were determined to be separate units of accounting, the related BESP, and the method of recognizing the allocated consideration upon each modification are as follows:

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Undelivered Element	BESP	Unit(s) of Accounting	Principal/Agent	Recognition Method
April 2015 modification				
AG-881 program licenses (1)	$33.2 million	2	Principal	Upon delivery of the licenses to Celgene, which occurred immediately upon the execution of the AG-881 Agreements
On-going development services (2) (3)	$12.7 million	4	Principal	Proportionally as services are delivered over the performance period, expected to be through 2017
On-going development services (2) (3)	$97.3 million	4	Agent	Proportionally as services are delivered over the performance period, expected to be through 2017
On-going research and development (2)	$30.5 million	1	Principal	Ratably over the performance period, expected to be through April 2016
Committee participations (4)	$0.8 million	1	Principal	Ratably over the performance period, expected to be through Q4 2016
May 2016 modification (supplants undelivered elements of April 2015 modification)				
On-going development services (2) (3)	$67.8 million	3	Principal	Proportionally as services are delivered over the performance period, expected to be through 2019
On-going development services (2) (3)	$22.4 million	3	Agent	Proportionally as services are delivered over the performance period, expected to be through 2019
On-going research and development (2)	$207.0 million	1	Principal	Ratably over the performance period, expected to be through May 2022
Committee participations (4)	$1.5 million	1	Principal	Ratably over the performance period, expected to be through Q4 2022
Additional development services	$48.7 million	1	Principal	Proportionally as services are delivered over the performance period, expected to begin in 2020

(1) The BESP was developed by probability weighting multiple cash flow scenarios using the income approach. Our management estimates within the models include the expected, probability-weighted net profits from estimated future sales, an estimate of the direct cost incurred to generate future cash flows, a discount rate and other business forecast factors. There are significant judgments and estimates inherent in the determination of the BESP of these units of accounting. These judgments and estimates include assumptions regarding future operating performance, the timelines of the clinical trials and regulatory approvals, and other factors. If different reasonable assumptions are utilized, the BESP and revenue recognized would vary.

(2) The BESP was developed using our management's best estimate of the cost of obtaining these services at arm's length from a third-party provider.

(3) The BESP was developed using internal full time equivalent costs to support the development services.

(4) The BESP was developed using our management's best estimate of the anticipated participation hours and the market rate for comparable participants.

The total estimated arrangement consideration, as well as the expected timing of revenue recognition, is adjusted based on changes in estimated arrangement consideration as a result of changes in estimates for on-going development services. The allocable consideration will increase as we perform certain services for which we are eligible to receive additional consideration. These amounts will be recognized on a cumulative catch-up basis for any in-process units of accounting or immediately for any fully delivered units of accounting. The estimated arrangement consideration may decrease if we receive less reimbursement than initially estimated.

During the three and six months ended June 30, 2017 and 2016, we recognized as collaboration revenue the following non-contingent consideration allocated to each undelivered element (in thousands):

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	Three Months Ended June 30,		Six Months Ended June 30,	
Undelivered Element	2017	2016	2017	2016
April 2015 modification				
AG-881 program licenses	$—	$503	$—	$1,356
On-going development services (principal)	—	623	—	1,656
On-going research and development	—	973	—	4,584
Committee participations	—	32	—	89
May 2016 modification				
On-going development services (principal)	3,866	1,663	7,612	1,663
On-going research and development	6,413	2,702	13,054	2,702
Committee participations	41	17	83	17
Total collaboration revenue	$10,320	$6,513	$20,749	$12,067

During the three and six months ended June 30, 2017 and 2016, we recognized as a reduction of research and development expenses the following non-contingent consideration allocated to each undelivered element (in thousands):

	Modification Period	Three Months Ended June 30,		Six Months Ended June 30,	
Undelivered Element		2017	2016	2017	2016
On-going development services (agent)	April 2015	$—	$2,636	$—	$7,456
On-going development services (agent)	May 2016	2,489	1,327	5,251	1,327
Total reduction of research and development expenses		$2,489	$3,963	$5,251	$8,783

Development and commercialization expenses that were not contemplated as of the modification dates due to the high level of uncertainty are recognized as collaboration revenue or a reduction of research and development expenses in the period in which they are earned. For the three and six months ended June 30, 2017 and 2016, we recognized the following collaboration revenue and reduction of research and development expenses related to such expenses (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Collaboration revenue - related party				
Development activities	$—	$465	$—	$1,192
Commercialization activities	$1,026	$—	$1,105	$—
Reduction of research and development expenses				
Development activities	$—	$1,959	$14	$5,933

During the three and six months ended June 30, 2017, we recognized a total of $11.3 million and $21.9 million, respectively, as collaboration revenue, and recognized $2.5 million and $5.3 million, respectively, as a reduction of research and development expenses. During the three and six months ended June 30, 2016, we recognized a total of $7.0 million and $13.3 million, respectively, as collaboration revenue, and recognized $5.9 million and $14.7 million, respectively, as a reduction of research and development expenses. As of June 30, 2017 and December 31, 2016, we recorded a collaboration receivable of $4.8 million and $4.9 million, respectively, related to reimbursable development costs.

We consider the total consideration expected to be earned in the next twelve months for services to be performed as current deferred revenue, and consideration that is expected to be earned subsequent to twelve months from the balance sheet date as noncurrent deferred revenue.

Accounting analysis and revenue recognition – milestone revenue

In January 2016, upon the initiation of the IDHENTIFY phase 3 study of IDHIFA®, we earned and received a milestone payment of $25.0 million, which was recognized as revenue during the three months ended March 31, 2016. No other milestones were earned during the six months ended June 30, 2017 and 2016. The next potential milestones

expected to be

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achieved under the Collaboration Agreements relate to specified ex-U.S. regulatory events. Achievement of these events will result in milestone payments of up to $70.0 million. Additionally, the next potential payment we expect to receive under the Collaboration Agreements is related to Celgene's exercise of its option to develop and commercialize AG-270, the program focused on MTAP-deleted cancers. This exercise may result in a license fee of at least $30.0 million.

Aurigene Discovery Technologies Limited

In April 2017, we entered into a new global license agreement with Aurigene Discovery Technologies Limited, or Aurigene, to research, develop and commercialize small molecule inhibitors for an undisclosed cancer metabolism target, or the Aurigene Agreement.

Under the terms of the Aurigene Agreement, Aurigene will provide to us exclusive rights to its portfolio of novel small molecules for the undisclosed target. Financial terms of the Aurigene Agreement include a $3.0 million upfront payment and potential future milestone payments of up to $17.0 million if we achieve certain development and regulatory milestones. Aurigene is also eligible to receive low single-digit royalties on product sales, if any. We will conduct preclinical studies and, if successful, fund further global research and development, as well as regulatory and commercial activities.

The term of the Aurigene Agreement will continue until the earlier of: (a) termination for convenience at our sole discretion upon 90 days prior written notice, (b) termination by either party for material breach, or (c) the expiration of the last-to-expire of all payment obligations hereunder with respect to all licensed products under the Aurigene Agreement.

Accounting analysis

The $3.0 million upfront payment was incurred in May 2017 and recorded as research and development expense. Costs incurred and milestones payments due to Aurigene prior to regulatory approval are recognized as expenses in the period incurred. Payments due to Aurigene upon or subsequent to regulatory approval will be capitalized and amortized over the shorter of the remaining license or product patent life.

6. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	June 30, 2017	December 31, 2016
Accrued compensation	$8,721	$ 11,092
Accrued research and development costs	17,714	20,266
Accrued professional fees	2,370	476
Accrued other	836	168
Total	$29,641	$ 32,002

7. Share-Based Payments

2013 Stock Incentive Plan

In June 2013, our Board of Directors adopted and, in July 2013 our stockholders approved, the 2013 Stock Incentive Plan, or the 2013 Plan. The 2013 Plan became effective upon the closing of our Initial Public Offering, or IPO, and provides for the grant of incentive stock options, non-qualified stock options, stock appreciation rights, restricted stock awards, restricted stock units, or RSUs, performance-based stock awards, or PSUs, and other stock-based awards. Following the adoption of the 2013 Plan, we granted no further stock options or other awards under the 2007 Stock Incentive Plan, or the 2007 Plan. Any options or awards outstanding under the 2007 Plan at the time of adoption of the 2013 Plan remain outstanding and effective. As of June 30, 2017, the total number of shares reserved under the 2007 Plan and the 2013 Plan are 7,660,664, and we had 1,293,777 shares available for future issuance under such plans.

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The following table presents stock option activity for the six months ended June 30, 2017:

	Number of Stock Options	Weighted-Average Exercise Price
Outstanding at December 31, 2016	5,218,880	$ 46.79
Granted	1,357,852	50.12
Exercised	(276,752)	20.85
Forfeited/expired	(233,817)	72.39
Outstanding at June 30, 2017	6,066,163	$ 47.73
Exercisable at June 30, 2017	3,004,850	$ 39.10
Vested and expected to vest at June 30, 2017	6,066,163	$ 47.73

At June 30, 2017, the total unrecognized compensation expense related to unvested stock option awards was $97.3 million, which we expect to recognize over a weighted-average period of approximately 2.5 years.

Restricted stock units

We may grant awards of RSUs to non-employee directors and employees on a discretionary basis pursuant to the 2013 Plan. Each RSU entitles the holder to receive, at the end of each vesting period, a specified number of shares of our common stock.

The following table presents RSU activity for the six months ended June 30, 2017:

	Number of Stock Units	Weighted-Average Grant Date Fair Value
Unvested shares at December 31, 2016	77,050	$ 49.60
Granted	57,100	50.40
Vested	(7,500)	122.22
Forfeited/expired	(7,300)	39.76
Unvested shares at June 30, 2017	119,350	$ 46.02

As of June 30, 2017, there was approximately $3.3 million of total unrecognized compensation expense related to RSUs, which we expect to be recognized over a weighted-average period of approximately 1.4 years.

Performance-based stock options

During the six months ended June 30, 2017 and 2016, no options to purchase shares of common stock that contain performance-based or a combination of performance-based and service-based vesting criteria were granted by us. However, certain performance-based stock options issued in prior periods were still outstanding as of June 30, 2017. Performance-based vesting criteria for options primarily relate to milestone events specific to our corporate goals, including but not limited to certain preclinical, clinical and regulatory development milestones related to our product candidates. Stock-based compensation expense associated with these performance-based stock options is recognized if the performance condition is considered probable of achievement using our management's best estimates. As of June 30, 2017, all performance-based milestones had been achieved and all expense related to these options have been recorded.

Performance-based stock units

We may grant awards of PSUs to non-employee directors and employees on a discretionary basis pursuant to the 2013 Plan. Each PSU entitles the holder to receive, at the achievement of the performance-based and service-based vesting criteria, a specified number of shares of our common stock. Performance-based vesting criteria primarily relate to milestone events specific to our corporate goals, specifically regulatory development milestones related to our product candidates.

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The following table presents PSU activity for the six months ended June 30, 2017:

	Number of Stock Units	Weighted-Average Grant Date Fair Value
Unvested shares at December 31, 2016	200,613	$ 53.36
Granted	6,391	48.72
Vested	—	—
Forfeited/expired	(25,630)	51.00
Unvested shares at June 30, 2017	181,374	$ 53.53

Stock-based compensation expense associated with these PSUs is recognized if the performance condition is considered probable of achievement using our management's best estimates. As of June 30, 2017, there was approximately $9.7 million of total unrecognized compensation expense related to PSUs with performance-based vesting criteria that are not considered probable of achievement.

2013 Employee Stock Purchase Plan

In June 2013, our Board of Directors adopted, and in July 2013 our stockholders approved, the 2013 Employee Stock Purchase Plan, or the 2013 ESPP. The 2013 ESPP is administered by our Board of Directors or by a committee appointed by our Board of Directors. Under the 2013 ESPP, each offering period is six months, at the end of which employees may purchase shares of common stock through payroll deductions made over the term of the offering period. The per-share purchase price at the end of each offering period is equal to 85% of the closing price of one share of our common stock at the beginning or end of the offering period, whichever is lower, subject to Internal Revenue Service limits. We issued 33,521 shares and 12,327 shares during the six months ended June 30, 2017 and 2016, respectively, under the 2013 ESPP. The 2013 ESPP provides participating employees with the opportunity to purchase up to an aggregate of 327,272 shares of our common stock. As of June 30, 2017, we had 239,921 shares available for future issuance under the 2013 ESPP.

Stock-based compensation expense

During the three and six months ended June 30, 2017 and 2016, we recorded stock-based compensation expense for employee and non-employee stock options, RSUs, performance-based stock options and ESPP shares. Stock-based compensation expense by award type included within the condensed consolidated statements of operations is as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Stock options	$11,083	$10,299	$20,985	$18,863
Restricted stock units	875	498	1,470	857
Performance-based stock options	—	—	—	46
Employee Stock Purchase Plan	229	199	466	337
Total stock-based compensation expense	$12,187	$10,996	$22,921	$20,103

Expenses related to equity-based awards were allocated as follows in the condensed consolidated statements of operations (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Research and development expense	$8,190	$6,561	$15,215	$12,089
General and administrative expense	3,997	4,435	7,706	8,014
Total stock-based compensation expense	$12,187	$10,996	$22,921	$20,103

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8. 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 the 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 dilutive net loss per share calculation, stock options, RSUs and ESPP options are considered to be common stock equivalents. Furthermore, performance-based stock units with vesting conditions that were not met as of June 30, 2017 are excluded as common stock equivalents.

Since we had a net loss for all periods presented, the effect of all potentially dilutive securities is anti-dilutive. Accordingly, basic and diluted net loss per share was the same for all periods presented.

The following common stock equivalents were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	Three and Six Months Ended June 30,	
	2017	2016
Stock options	6,066,163	5,383,189
Restricted stock units	119,350	66,300
Employee stock purchase plan options	18,287	15,917
Total common stock equivalents	6,203,800	5,465,406

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

Forward-looking Information

The following discussion of our financial condition and results of operations should be read with our unaudited condensed consolidated financial statements as of June 30, 2017 and 2016 and for the three and six months ended June 30, 2017 and 2016, and related notes included in Part I. Item 1 of this Quarterly Report on Form 10-Q, as well as the audited consolidated financial statements and notes and Management's Discussion and Analysis of Financial Condition and Results of Operations, included in our Annual Report on Form 10-K for the year ended December 31, 2016 filed with the Securities and Exchange Commission, or SEC, on February 16, 2017. This Management's Discussion and Analysis of Financial Condition and Results of Operations contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements are based on current expectations, estimates, forecasts and projections, and the beliefs and assumptions of our management, and include, without limitation, statements with respect to our expectations regarding our research, development and commercialization plans and prospects, results of operations, general and administrative expenses, research and development expenses, and the sufficiency of our cash for future operations. Words such as "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "c statements or variation of these terms or the negative of those terms and similar expressions are intended to identify these forward-looking statements. Readers are cautioned that these forward-looking statements are predictions and are subject to risks, uncertainties and assumptions that are difficult to predict. Therefore, actual results may differ materially and adversely from those expressed in any forward-looking statements. Among the important factors that could cause actual results to differ materially from those indicated by our forward-looking statements are those discussed under the heading "Risk Factors" in Part II, Item 1A and elsewhere in this report, and in our Annual Report on Form 10-K. We undertake no obligation to revise the forward-looking statements contained herein to reflect events or circumstances after the date hereof or to reflect the occurrence of unanticipated events.

Overview

We are a biopharmaceutical company committed to the fundamental transformation of patients' lives through scientific leadership in the field of cellular metabolism, with the goal of making transformative, first- or best-in-class medicines. Our areas of focus are cancer metabolism, rare genetic diseases, or RGDs, which are diseases that are directly caused by changes in genes or chromosomes, often passed from one generation to the next, and metabolic immuno-oncology, or MIO, which is a developing field that aims to modulate the activity of relevant immune cells (or tumor microenvironment) by targeting critical metabolic nodes, thereby enhancing the immune mediated anti-tumor response. In each of these areas, we are seeking to unlock the biology of cellular metabolism as a platform to create transformative therapies.

On August 1, 2017, the U.S. Food and Drug Administration, or FDA, granted our collaboration partner Celgene Corporation, or Celgene, approval of IDHIFA® for the treatment of adult patients with relapsed or refractory acute myeloid leukemia, or R/R AML, with an isocitrate dehydrogenase 2, or IDH2, mutation as detected by an FDA-approved test. IDHIFA®, an oral targeted inhibitor of the IDH2 enzyme, is the first and only FDA-approved therapy for patients with R/R AML and an IDH2 mutation.

Our most advanced cancer product candidates are ivosidenib, which targets mutated isocitrate dehydrogenase 1, or IDH1, and AG-881, a brain-penetrant, pan-IDH mutant inhibitor, which provides added flexibility to our current portfolio of IDH mutant inhibitors.

The lead product candidate in our RGD program, AG-348, targets pyruvate kinase-R for the treatment of pyruvate kinase, or PK, deficiency. PK deficiency is a rare genetic disorder that often results in severe hemolytic anemia, jaundice and lifelong conditions associated with chronic anemia, and secondary complications due to inherited mutations in the pyruvate kinase enzyme within red blood cells.

2016 Agreement

In May 2016, we entered into a master research and collaboration agreement, or the 2016 Agreement, with Celgene Corporation, or Celgene, and Celgene RIVOT Ltd., a wholly owned subsidiary of Celgene. The 2016 Agreement

focuses on the discovery and development of cancer programs in the field of MIO. In addition to new programs identified under the 2016 Agreement, both parties also agreed that all future development and commercialization of two remaining cancer metabolism programs discovered under the 2010 discovery and development collaboration and license agreement with Celgene, or the 2010 Agreement, including AG-270, a program focused on methylthioadenosine phosphorylase, or MTAP, deleted cancers, will now be governed by the 2016 Agreement.

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During the research term of the 2016 Agreement, we plan to conduct research programs focused on discovering compounds that are active against metabolic targets in the immuno-oncology, or IO, field. The initial four-year research term will expire on May 17, 2020, and may be extended for up to two, or in specified cases, up to four additional one-year terms.

For each program under the 2016 Agreement, we may nominate compounds that meet specified criteria as development candidates and, in limited circumstances, Celgene may also nominate compounds as development candidates for each such program. Celgene may designate the applicable program for further development following any such nomination, after which we may conduct, at our expense, additional preclinical and clinical development for such program through the completion of an initial phase 1 dose escalation study.

At the end of the research term, Celgene may designate for continued development up to three research programs for which development candidates have yet to be nominated, which are referred to as continuation programs. We may conduct further research and preclinical and clinical development activities on any continuation program, at our expense, through the completion of an initial phase 1 dose escalation study.

We granted Celgene the right to obtain exclusive options for development and commercialization rights for each program that Celgene has designated for further development, and for each continuation program. Celgene may exercise each such option beginning on the designation of a development candidate for such program (or on the designation of such program as a continuation program) and ending on the earlier of: (i) the end of a specified period after we have furnished Celgene with specified information about the initial phase 1 dose escalation study for such program, or (ii) January 1, 2030. Research programs that have applications in the inflammation or autoimmune, or I&I, field that may result from the 2016 Agreement will also be subject to the exclusive options described above. We will retain rights to any program that Celgene does not designate for further development or as to which it does not exercise its option.

Under the terms of the 2016 Agreement, following Celgene's exercise of its option with respect to a program, the parties will enter into either a co-development and co-commercialization agreement if such program is in the IO field, or a license agreement if such program is in the I&I field. Under each co-development and co-commercialization agreement, the two parties will co-develop and co-commercialize licensed products worldwide. Either we or Celgene will lead development and commercialization of licensed products for the United States, and Celgene will lead development and commercialization of licensed products outside of the United States. Depending on the country, the parties will each have the right to provide a portion of field-based marketing activities. Under each license agreement, Celgene will have the sole right to develop and commercialize licensed products worldwide.

AG-120 Letter Agreement

On May 17, 2016, we entered into a letter agreement with Celgene regarding ivosidenib, or the AG-120 Letter Agreement. Under the AG-120 Letter Agreement, the parties agreed to terminate the 2010 Agreement, effective as of August 15, 2016, as to the program directed to the IDH1 target, for which ivosidenib is the lead development candidate. Under the 2010 Agreement, Celgene had held development and commercialization rights to the IDH1 program outside of the United States, and we held such rights inside the United States. As a result of the termination, we obtained global rights to ivosidenib and the IDH1 program. Neither party will have any further financial obligation, including royalties or milestone payments, to the other concerning ivosidenib or the IDH1 program. Under the terms of the termination, the parties have also agreed to conduct specified transitional activities in connection with the termination. In addition, pursuant to the AG-120 Letter Agreement, the parties are released from their exclusivity obligations under the 2010 Agreement with respect to the IDH1 program. The termination does not affect the AG-881 Agreements described below, which are directed to both the IDH1 target and the IDH2 target.

AG-881 Agreements

On April 27, 2015, we entered into a joint worldwide development and profit share collaboration and license agreement with Celgene, and our wholly owned subsidiary, Agios International Sarl, entered into a collaboration and license agreement with Celgene International II Sarl. Both of these agreements are collectively referred to as the AG-881 Agreements. The AG-881 Agreements establish a joint worldwide collaboration focused on the development and commercialization of AG-881 products. Under the terms of the AG-881 Agreements, we received an initial upfront payment of $10.0 million in May 2015 and are eligible to receive up to $70.0 million in milestone-based

payments. The parties will split all worldwide development costs equally, subject to specified exceptions, as well as any profits from any net sales of, or commercialization losses related to, licensed AG-881 products.

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2010 Agreement

In April 2010, we entered into the 2010 Agreement, which was amended in October 2011 and July 2014. The goal of the collaboration was to discover, develop and commercialize disease-altering therapies in oncology based on our cancer metabolism research platform. We initially led discovery, preclinical and early clinical development for all cancer metabolism programs under the collaboration. The discovery phase of the 2010 Agreement expired in April 2016.

Upon agreement to terminate the 2010 Agreement, effective as of August 15, 2016, as to the program directed to the IDH1 target, for which ivosidenib is the lead development candidate, the sole program remaining under the 2010 Agreement is IDHIFA®, a co-commercialized licensed program for which Celgene leads and funds global development and commercialization activities. We have exercised our right to participate in a portion of commercialization activities in the United States for IDHIFA® in accordance with the applicable commercialization plan. On August 1, 2017, the FDA granted Celgene approval of IDHIFA® for the treatment of adult patients with R/R AML and an IDH2 mutation as detected by an FDA-approved test. We are eligible to receive up to $95.0 million in potential milestone-based payments for the IDHIFA® program, which are comprised of: (i) up to $70.0 million in milestone payments upon achievement of specified ex-U.S. regulatory milestone events and (ii) a $25.0 million milestone payment upon achievement of a specified commercial milestone event. Additionally, we are eligible to receive tiered royalties on any net sales of IDHIFA®.

Critical Accounting Policies and Estimates

Our critical accounting policies are those policies which require the most significant judgments and estimates in the preparation of our consolidated financial statements. We have determined that our most critical accounting policies are those relating to revenue recognition, accrued research and development expenses and stock-based compensation. There have been no significant changes to our critical accounting policies discussed in the Annual Report on Form 10-K for the year ended December 31, 2016.

Financial Operations Overview

General

Since inception, our operations have focused on organizing and staffing our company, business planning, raising capital, assembling our core capabilities in cellular metabolism, identifying potential product candidates, undertaking preclinical studies and conducting clinical trials. To date, we have financed our operations primarily through funding received from the 2010 Agreement, the AG-881 Agreements, the 2016 Agreement, private placements of our preferred stock, our initial public offering of our common stock and concurrent private placement of common stock to an affiliate of Celgene, and our follow-on public offerings.

Additionally, since inception, we have incurred significant operating losses. Our net losses were $149.2 million and $79.2 million for the six months ended June 30, 2017 and 2016, respectively. As of June 30, 2017, we had an accumulated deficit of $632.6 million. We expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from year to year. We anticipate that our expenses will increase significantly as we continue to advance and expand clinical development activities for our lead programs, IDHIFA®, ivosidenib, AG-881 and AG-348; continue to discover and validate novel targets and drug product candidates; expand and protect our intellectual property portfolio; and hire additional commercial, development and scientific personnel.

Revenue

Through June 30, 2017, we have not generated any revenue from product sales. Substantially all of our revenue to date has been derived from our collaborations. In the future, we expect to generate revenue from a combination of product sales, upfront payments, cost reimbursements, milestone payments, and royalties on future product sales.

Research and development expenses

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress. However, the successful development of our product candidates is highly uncertain. As such, at this time, we cannot reasonably

estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the remainder of the development and commercialize these product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from IDHIFA®, ivosidenib, AG-881, AG-348, AG-270 or any of our other product candidates. This is due to the numerous risks and uncertainties associated with developing medicines, including the uncertainty of:

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- establishing an appropriate safety profile with investigational new drug application, or IND, and/or new drug application, or NDA, enabling toxicology and clinical studies;
- the successful enrollment in, and completion of, clinical trials;
- the receipt of marketing approvals from applicable regulatory authorities;
- establishing compliant commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection, and regulatory exclusivity for our product candidates;
- launching commercial sales of the products, if and when approved, whether alone or in collaboration with others; and
- maintaining an acceptable safety profile of the products following approval.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate.

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

- employee-related expenses, including salaries, benefits and stock-based compensation expense;
- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct research and development and both preclinical and clinical activities on our behalf, and the cost of consultants;
- the cost of lab supplies and acquiring, developing and manufacturing preclinical and clinical study materials; and
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and the maintenance of facilities, insurance and other operating costs.

The following summarizes our most advanced current research and development programs:

IDHIFA®

IDHIFA® is an orally available, selective, potent inhibitor of the mutated IDH2 protein, making it a highly targeted therapeutic candidate for the treatment of patients with cancers that harbor IDH2 mutations, including those with AML, who have a historically poor prognosis. On August 1, 2017, the FDA granted Celgene approval of IDHIFA® for the treatment of adult patients with R/R AML and an IDH2 mutation as detected by an FDA-approved test.

Celgene maintains worldwide development and commercial rights to IDHIFA® and will fund the future development and commercialization costs related to this program. Under the 2010 Agreement, Celgene is responsible for all development costs for IDHIFA®, and we are eligible to receive up to $95.0 million in milestone payments, which are comprised of: (i) up to $70.0 million in milestone payments upon achievement of specified ex-U.S. regulatory milestone events and (ii) a $25.0 million milestone payment upon achievement of a specified commercial milestone event. Additionally, we are eligible to receive tiered royalties on any net sales of IDHIFA®. In January 2016, we earned a $25.0 million milestone payment upon initiation of the IDHENTIFY clinical trial, as described below. In addition to contributing our scientific and translational expertise, we will continue to conduct some clinical development and regulatory activities within the IDHIFA® development program under the 2010 Agreement. We also have co-commercialization rights to provide up to one third of the commercialization efforts and will be reimbursed for those efforts.

We continue to evaluate IDHIFA® in clinical trials evaluating hematological cancers with IDH2 mutations, which are led and funded by Celgene. To date, all clinical data reported by us and our collaborators in hematological cancers highlights that the mechanism of response is consistent with preclinical studies, including substantial reduction of plasma 2-hydroxygluturate, or 2HG, levels, as well as evidence of cellular differentiation and normalization of cell counts in the bone marrow and blood. This differentiation effect is distinct from that seen with traditional chemotherapeutics commonly used to treat AML. The FDA has

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granted orphan drug designation for IDHIFA® for treatment of patients with AML and fast track designation for treatment of patients with AML that harbor an IDH2 mutation, and the European Medicines Agency, or EMA, granted orphan drug designation for IDHIFA® for the treatment of AML.

We and Celgene are evaluating IDHIFA® in the following clinical trials:

Phase 1/2 clinical trial

IDHIFA® is being evaluated in a phase 1/2 multicenter, open-label, clinical trial, initially conducted by us but which Celgene has now assumed primary responsibility for, to assess the safety, clinical activity, and tolerability of IDHIFA® in patients with advanced hematologic malignancies with an IDH2 mutation.

Together with Celgene, we presented clinical data from a subset of patients with myelodysplastic syndromes, or MDS, from the dose-escalation and expansion portions of this trial at the 2016 American Society of Hematology Annual Meeting and Exposition in San Diego, California, or ASH 2016, in December 2016.

In June 2017, we and Celgene presented clinical data from the phase 1 dose-escalation and expansion portions of this trial in R/R AML at the American Society of Clinical Oncology, or ASCO 2017, in Chicago, Illinois. Also in June 2017, we and Celgene presented updated clinical data from this trial, including data from the phase 2 portion of the trial, at the 22nd Congress of the European Hematology Association, or 2017 EHA, in Madrid, Spain.

Phase 1 frontline combination trial (ivosidenib also being evaluated)

IDHIFA® is being evaluated in a phase 1, multicenter, international, open-label clinical trial, conducted by us, to evaluate the safety and clinical activity of IDHIFA® or ivosidenib in combination with induction and consolidation therapy in patients with newly diagnosed AML with an IDH2 or IDH1 mutation who are eligible for intensive chemotherapy. The trial is currently enrolling patients.

Phase 1/2 frontline combination trial (ivosidenib also being evaluated)

IDHIFA® is being evaluated in a phase 1/2 frontline combination clinical trial, conducted by Celgene, of either IDHIFA® or ivosidenib in combination with VIDAZA® (azacitidine) in newly diagnosed AML patients not eligible for intensive chemotherapy, with a phase 1 component to determine the safety of the combinations, followed by a phase 2 randomized component evaluating the safety and clinical activity of each investigational combination versus single-agent VIDAZA® using a primary endpoint of overall response rate. The trial has completed the phase 1 component and is currently enrolling in the phase 2 component.

IDHENTIFY

IDHIFA® is being evaluated in IDHENTIFY, an international phase 3, multi-center, open-label, randomized clinical trial, conducted by Celgene, designed to compare the efficacy and safety of IDHIFA® versus conventional care regimens in patients 60 years or older with IDH2 mutant-positive AML that is refractory to or relapsed after second- or third-line therapy. In January 2016, in conjunction with the initiation of the IDHENTIFY clinical trial, we received a milestone payment of $25.0 million from Celgene pursuant to the 2010 Agreement. The trial is currently enrolling patients.

Ivosidenib

Ivosidenib is an orally available, selective, potent inhibitor of the mutated IDH1 protein, making it a highly targeted therapeutic candidate for the treatment of patients with cancers that harbor IDH1 mutations. We hold worldwide development and commercial rights to ivosidenib and will fund the future development and commercialization costs related to this program. Mutations in IDH1 have been identified in difficult to treat hematologic and solid tumor cancers, including AML, chondrosarcoma, cholangiocarcinoma, and glioma, where both the treatment options and prognosis for patients are poor. On May 18, 2015, we announced that the FDA granted fast track designation to ivosidenib for treatment of patients with AML that harbor an IDH1 mutation. On June 10, 2015, the FDA granted us orphan drug designation for ivosidenib for treatment of patients with AML. In November 2016, the FDA granted fast track designation to ivosidenib for treatment of patients with previously treated, unresectable or metastatic cholangiocarcinoma with an IDH1 mutation. In January 2017, we announced that we intend to submit an NDA to the FDA for ivosidenib in R/R AML by the end of 2017. On April 26, 2017, the FDA granted orphan drug designation for ivosidenib for the treatment of cholangiocarcinoma.

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We are evaluating ivosidenib in the following clinical trials:

Phase 1 clinical trial (advanced hematologic malignancies)

Ivosidenib is being evaluated in a phase 1 multicenter, open-label, dose-escalation and expansion clinical trial, designed to assess its safety, clinical activity and tolerability as a single agent in patients with advanced hematologic malignancies with an IDH1 mutation. Four expansion cohorts have been added to the trial.

In December 2016, we presented clinical data from 78 patients treated with ivosidenib in the completed dose escalation portion of the ongoing phase 1 study of ivosidenib in advanced hematologic malignancies at ASH 2016. This was the first demonstration that treatment with single agent ivosidenib can result in mutant IDH1 clearance, or IDH1-MC. We are continuing to study the potential relationship between IDH1-MC and the clinical benefit for patients with AML.

Phase 1 frontline combination trial

As discussed above, ivosidenib and IDHIFA® are also being evaluated in a phase 1, multicenter, international, open-label clinical trial, in combination with induction and consolidation therapy. The trial is currently enrolling patients.

Phase 1/2 frontline combination trial

As discussed above, ivosidenib and IDHIFA® are also being evaluated in a phase 1/2 frontline clinical trial in combination with VIDAZA®, conducted by Celgene. The trial has completed the phase 1 component and is currently enrolling in the phase 2 component.

AGILE

Ivosidenib is being evaluated in AGILE, a global, registration-enabling phase 3 clinical trial, combining ivosidenib and VIDAZA® in newly diagnosed AML patients with an IDH1 mutation who are ineligible for intensive chemotherapy. The trial is currently open for enrollment.

Phase 1 clinical trial (advanced solid tumors)

Ivosidenib is being evaluated in a phase 1 multicenter, open-label, dose-escalation and expansion clinical trial, designed to assess its safety, clinical activity and tolerability as a single agent in patients with advanced solid tumors with an IDH1 mutation, including glioma, intrahepatic cholangiocarcinoma, or IHCC, and chondrosarcomas.

In November 2016, we reported initial data from the dose expansion cohort of our ongoing phase 1 clinical trial evaluating ivosidenib in patients with IDH1 mutant-positive glioma at the Society for Neuro-Oncology Annual Meeting in Scottsdale, Arizona, and initial data from the dose expansion cohort of our ongoing phase 1 clinical trial evaluating ivosidenib in patients with IDH1 mutant-positive chondrosarcoma at the annual meeting of the Connective Tissue Oncology Society, or CTOS, in Lisbon, Portugal.

In June 2017, we presented updated clinical data from the dose-escalation and expansion cohorts of our ongoing phase 1 study evaluating ivosidenib in patients with IDH1 mutant-positive cholangiocarcinoma at ASCO 2017.

ClarIDHy

Ivosidenib is being evaluated in ClarIDHy, a registration-enabling phase 3, multicenter, randomized, double-blind, placebo-controlled clinical trial of ivosidenib in previously-treated patients with nonresectable or metastatic cholangiocarcinoma with an IDH1 mutation. The trial was initiated in December 2016 and is currently enrolling patients.

AG-881: brain penetrant pan-IDH program

AG-881 is an orally available, selective, brain-penetrant, pan-IDH mutant inhibitor, which provides added flexibility to our current portfolio of IDH mutant inhibitors. Given the clinical data generated with IDHIFA® and ivosidenib in AML, AG-881 will remain a back-up molecule in hematologic malignancies, and we are focusing our development efforts for AG-881 in glioma.

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We and Celgene are jointly collaborating on a worldwide development program for AG-881, wherein we share worldwide development costs and profits and Celgene would book any worldwide commercial sales. We will lead commercialization in the United States with both companies sharing equally in field-based commercial activities, and Celgene will lead commercialization outside of the United States with us providing one third of field-based commercial activities in the major EU markets. Under the AG-881 Agreements, we are eligible to receive up to $70.0 million in potential milestone payments related to AG-881. We may also receive royalties at tiered, low-double digit to mid-teen percentage rates on net sales if we elect not to participate in the development and commercialization of AG-881.

We are conducting two phase 1 multi-center, open-label clinical trials of AG-881, one in patients with advanced IDH1 or IDH2 mutant-positive solid tumors, including glioma, and the other in patients with advanced IDH1 or IDH2 mutant-positive hematologic malignancies whose cancer has progressed on a prior IDH inhibitor therapy. The goal of these trials is to evaluate the safety, pharmacokinetics, pharmacodynamics and clinical activity of AG-881 in advanced solid tumors and hematologic malignancies, respectively.

The phase 1 trial in patients with advanced IDH1 or IDH2 mutant-positive hematologic malignancies has completed its dose escalation portion, establishing proof of mechanism as measured by reductions in 2HG levels, and is now closed for enrollment. No maximum tolerable dose, or MTD, was reached. In the phase 1 trial in IDH1 or IDH2 mutant-positive advanced solid tumors, including glioma, the dose escalation portion has completed enrollment. Although MTD has not been reached for this trial, additional patients will be enrolled for additional safety, pharmacokinetics and pharmacodynamics analyses.

AG-348: PKR activator

AG-348 is an orally available small molecule and a potent activator of the wild-type (normal) and mutated PKR enzyme, which has resulted in restoration of adenosine triphosphate, or ATP, levels and a decrease in 2,3-diphosphoglycerate, or 2,3-DPG, levels in blood sampled from patients with PK deficiency and treated ex-vivo with AG-348. The wild-type PKR activity of AG-348 allowed the study of enzyme activation in healthy volunteers, providing an opportunity to understand the safety, dosing and pharmacodynamic activity of AG-348 prior to entering a proof-of-concept study in patients. We have worldwide development and commercial rights to AG-348 and expect to fund the future development and commercialization costs related to this program. On March 24, 2015, the FDA granted us orphan drug designation for AG-348 for treatment of patients with PK deficiency. In December 2016, we announced our decision to advance AG-348 into pivotal development as the first potential disease-modifying treatment for PK deficiency. On April 27, 2017, the FDA granted fast track designation to AG-348 for the treatment of patients with PK deficiency.

DRIVE PK

In June 2015, we initiated DRIVE PK, a global phase 2, first-in-patient, open-label safety and efficacy clinical trial of AG-348 in adult, transfusion-independent patients with PK deficiency.

In June 2016, we reported the first clinical data from DRIVE PK at the 21st Congress of EHA in Copenhagen, Denmark, establishing proof of concept for AG-348 as a novel, first-in-class, oral activator of both wild-type and mutated PKR enzymes.

This trial has reached target enrollment, and in June 2017 we presented updated clinical data from DRIVE PK at 2017 EHA.

AG-270: Targeting MTAP-deleted cancers

AG-270 is our development candidate focused on MTAP-deleted cancers. In March 2017, we announced that Celgene designated AG-270 as a development candidate under the 2016 Agreement. Pursuant to the 2016 Agreement, Celgene paid us an $8.0 million designation fee for AG-270. Exploratory research, drug discovery and early development of AG-270 is led by us, and Celgene will have an opt-in right on AG-270 up through phase 1 dose escalation for at least a $30.0 million fee. Upon opt-in, we and Celgene will have global co-development and co-commercialization rights with a worldwide 50/50 cost and profit share on AG-270, and we will be eligible for up to $168.8 million in clinical and regulatory milestone payments. We expect to submit an IND for AG-270 by the end of 2017.

MTAP is a metabolic enzyme that is deleted in approximately 15 percent of all cancers. This deletion is readily detected by a simple genomic or immunohistochemistry test, thus allowing the selection of patients predicted to be

sensitive to the therapy. We have discovered a novel pathway comprised of multiple targets with a shared vulnerability in MTAP-deleted tumors and have demonstrated that this pathway can be modulated by small molecule inhibitors, resulting in robust anti-tumor activity in animal models.

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AG-519

AG-519 is an orally available small molecule and a potent activator of the PKR enzyme, with comparable biochemical, cellular and in-vivo activity to AG-348, and was developed as our second PKR activator. We were evaluating AG-519 in a placebo-controlled phase 1 integrated single ascending dose and multiple ascending dose clinical trial in healthy volunteers. In December 2016, we announced that we are no longer developing AG-519, and withdrew our IND for AG-519, following a verbal notification of a clinical hold from the FDA.

Other research and platform programs

Other research and platform programs include activities related to exploratory efforts, target validation and lead optimization for our discovery and follow-on programs, and our proprietary metabolomics platform.

General and administrative expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development, legal and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters, and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities and potential commercialization of our product candidates. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses.

Results of Operations

Comparison of the three months ended June 30, 2017 and 2016

The following table summarizes our results of operations for the three months ended June 30, 2017 and 2016:

	Three Months Ended June 30,			
($ in thousands)	2017	2016	$ Change	% Change
Collaboration revenue – related party	$11,346	$6,978	$4,368	63 %
Operating expenses:				
Research and development (net of $2,489 and $5,922 of cost reimbursement from related party for the three months ended June 30, 2017 and 2016, respectively)	79,816	50,804	29,012	57 %
General and administrative	16,130	12,644	3,486	28 %
Loss from operations	(84,600)	(56,470)	(28,130)	50 %
Interest income	1,518	517	1,001	194 %
Net loss	$(83,082)	$(55,953)	$(27,129)	48 %

Revenue. For the three months ended June 30, 2017, we recognized $11.3 million in revenue, which includes $10.3 million related to deliverables identified under the 2016 Agreement.

For the three months ended June 30, 2016, we recognized $7.0 million in revenue, which includes $4.4 million related to deliverables identified under the 2016 Agreement and does not include any revenue associated with the ivosidenib program after the May 2016 modification date.

Research and Development Expense. The increase in research and development expenses was primarily attributable to net increases of $24.5 million in external services and $4.5 million in internal expenses; both of these increases are inclusive of cost reimbursements recorded as a reduction of research and development expenses.

We use our employee and infrastructure resources across multiple research and development programs, and we allocate internal employee-related and infrastructure costs, including stock-based compensation and facilities costs, as well as certain third-party costs, net of reimbursements from Celgene, to our research and development programs based on the personnel resources

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allocated to such program. Our allocated research and development expenses, by major program, are outlined in the table below:

	Three Months Ended June 30,			
($ in thousands)	2017	2016	$ Change	% Change
IDH2 inhibitor (IDHIFA®)	$1,885	$2,783	$ (898)	(32)%
IDHIFA® reduction of R&D expenses	—	(704)	704	(100)%
IDH1 inhibitor (ivosidenib)	38,883	23,854	15,029	63 %
Ivosidenib reduction of R&D expenses (1)	—	(3,282)	3,282	(100)%
Pan-IDH inhibitor (AG-881)	5,622	5,065	557	11 %
AG-881 reduction of R&D expenses	(2,489)	(1,936)	(553)	29 %
PKR activator (AG-348)	10,821	5,099	5,722	112 %
MTAP-deleted cancers program (AG-270)	5,084	4,448	636	14 %
Discontinued backup PKR activator (AG-519)	1,589	4,737	(3,148)	(66)%