



## **Sarepta Therapeutics Announces Second Quarter 2019 Financial Results and Recent Corporate Developments**

CAMBRIDGE, Mass., August 7, 2019 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the second quarter of 2019.

“As we pass through mid-2019, we are very pleased to announce strong performance and solid execution against our goals. EXONDYS 51®(eteplirsen) continues to perform, with second quarter sales at \$94.7 million, 29% growth quarter over same quarter last year. In our RNA franchise, we advanced our VYONDYS 53™(golodirsen) application, with an FDA PDUFA date of August 19, are preparing to submit for casimersen, with a target PDUFA date in the first half of 2020, and have commenced dosing our PPMO SRP-5051 MAD trial. With respect to our gene therapy platform, we have completed the dosing of the 24 patients in our placebo-controlled micro-dystrophin trial as forecasted, made significant progress in the build out of our commercial micro-dystrophin process and manufacturing facility, advanced our gene therapy engine, and through internal development and partnering have added a number of new programs to our pipeline. Beyond the LGMD 2A program announced during the quarter, in collaboration with industry leading gene therapy experts we are now pursuing additional gene therapies for Rett Syndrome, cardiomyopathy, Emery-Dreifuss muscular dystrophy type 1, and, moving outside of rare disease, multiple sclerosis,” stated Doug Ingram, Sarepta’s President and CEO.

Mr. Ingram continued, “With respect to our most advanced gene therapy programs, we are in a privileged leadership position in 2019, both from our own execution and from the evolution of external programs. That privilege, however, comes with an enhanced responsibility to the patient community we serve. We have much to do in the second half of 2019, and we intend to perform with the same level of attention to detail and urgency as has become our reputation.”

### **Second Quarter 2019 and Recent Corporate Developments**

- **First Patient Dosed in MOMENTUM, a Phase 2 Clinical Trial Investigating SRP-5051:** The first patient was dosed in MOMENTUM (Study 5051-201), a global Phase 2 clinical trial of SRP-5051, a

next-generation treatment for patients with Duchenne muscular dystrophy who are amenable to exon 51 skipping. SRP-5051 is the first investigational treatment using Sarepta's next-generation PPMO platform, which is designed around a proprietary cell-penetrating peptide conjugated to the phosphorodiamidate morpholino oligomer (PMO) backbone with the goal of increasing drug concentration in muscle tissue. MOMENTUM is a multi-arm, ascending dose study designed to identify the maximum tolerated dose of SRP-5051. Informed by Study 5051-101, a single-ascending dose study of SRP-5051, patients in the MOMENTUM study will receive monthly intravenous (IV) infusions of SRP-5051. The study will enroll up to 24 patients, both ambulant and non-ambulant, between the ages of 7-21 at sites in the U.S., Canada, Australia and European Union. Primary and secondary endpoints include dystrophin expression and functional and clinical endpoints.

- **Dosed 24 Patients in Study 102:** 24 patients have been dosed in Study SRP-9001-102, a blinded, placebo-controlled trial using Sarepta's micro-dystrophin gene therapy candidate for Duchenne muscular dystrophy, SRP-9001.
- **Conditional Approval of the Brand Name for Golodirsen (SRP-4053) Received by U.S. Food and Drug Administration:** Sarepta received FDA's conditional approval of VYONDYS 53™ as the brand name for golodirsen. VYONDYS 53 (golodirsen) is Sarepta's phosphorodiamidate morpholino oligomer (PMO) engineered to treat individuals with Duchenne muscular dystrophy who have genetic mutations amenable to skipping exon 53 of the dystrophin gene.

### **Conference Call**

The Company will be hosting a conference call at 4:30 p.m. Eastern Time to discuss Sarepta's financial results and provide a corporate update. The conference call may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 for international callers. The passcode for the call is 5789017. Please specify to the operator that you would like to join the "Sarepta Second Quarter 2019 Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at [www.sarepta.com](http://www.sarepta.com) and will be archived there following the call for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

### **Financial Results**

On a GAAP basis, the Company reported a net loss of \$276.4 million and \$109.3 million, or \$3.74 and \$1.67 per basic and diluted share for the second quarter of 2019 and 2018, respectively. On a non-GAAP basis,

the net loss for the second quarter of 2019 was \$61.2 million, or \$0.83 per basic and diluted share, compared to a net loss of \$28.0 million for the same period of 2018, or \$0.43 per basic and diluted share.

On a GAAP basis, for the six months ended June 30, 2019, Sarepta reported a net loss of \$353.0 million, or \$4.85 per basic and diluted share, compared to a net loss of \$144.6 million reported for the same period of 2018, or \$2.22 per basic share and diluted share. On a non-GAAP basis, the net loss for the six months ended June 30, 2019 was \$115.0 million, or \$1.58 per share, compared to a net loss of \$46.0 million for the same period of 2018, or \$0.71 per share.

### ***Net Revenues***

For the three months ended June 30, 2019, the Company recorded net revenues of \$94.7 million, compared to net revenues of \$73.5 million for the same period of 2018, an increase of \$21.2 million. For the six months ended June 30, 2019, the Company recorded net revenues of \$181.7 million, compared to net revenues of \$138.1 million for six months ended June 30, 2018, an increase of \$43.5 million. The increases primarily reflect the continuing increase in demand for EXONDYS 51 in the U.S.

### ***Cost and Operating Expenses***

#### *Cost of sales (excluding amortization of in-licensed rights)*

For the three months ended June 30, 2019, cost of sales (excluding amortization of in-licensed rights) was \$15.9 million, compared to \$6.7 million for the same period of 2018. For the six months ended June 30, 2019, cost of sales (excluding amortization of in-licensed rights) was \$28.0 million, compared to \$12.3 million for the same period of 2018. The increase primarily reflects royalty payments to BioMarin Pharmaceuticals (BioMarin) and University of Western Australia (UWA), and higher product costs as a result of increasing demand for EXONDYS 51, as well as an inventory write-off related to certain batches of product not meeting the Company's quality specifications. Prior to the approval of EXONDYS51, the Company expensed related manufacturing and material costs as research and development expenses. As a result, the Company sold more product with no cost during the six months ended June 30, 2018 compared with the same period of 2019.

#### *Research and development*

Research and development expenses were \$113.3 million for the second quarter of 2019, compared to \$122.8 million for the same period of 2018, a decrease of \$9.6 million. The decrease in research and development expenses primarily reflects the following:

- \$44.9 million decrease in up-front and milestone payments primarily due to \$14.4 million of up-front payments as a result of execution of certain license agreements during the second quarter

of 2019, offset by an up-front payment of \$60.0 million to Myonex upon execution of the warrant to purchase common stock agreement in May 2018;

- \$2.7 million decrease in collaboration cost sharing with Summit as it is winding down activities on its Utrophin platform;
- \$2.2 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in our PPMO platform;
- \$16.5 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of our micro-dystrophin program, our ESSENCE program and initiation of certain post-market studies for EXONDYS 51. The increases were partially offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- \$9.9 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- \$7.4 million increase in facility- and technology-related expenses due to our continuing expansion efforts as well as change in methodology in allocation of technology expense;
- \$1.9 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price; and
- \$3.4 million increase in research and other primarily driven by an increase in lab supplies as a result of an increase in headcount as well as sponsored research with academic institutions.

Research and development expenses were \$203.8 million for the six months ended June 30, 2019, compared to \$169.1 million for the same period of 2018, an increase of \$34.8 million. The increase in research and development expenses primarily reflects the following:

- \$35.3 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of our micro-dystrophin program, our ESSENCE program and initiation of certain post-market studies for EXONDYS 51. The increases were partially offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- \$21.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- \$15.7 million increase in facility- and technology-related expenses due to our continuing expansion efforts as well as change in methodology in allocation of technology expense;

- \$4.9 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- \$43.8 million decrease in up-front and milestone payments primarily due to \$15.5 million of up-front payments as a result of license agreements executed during the second quarter of 2019, offset by an up-front payment of \$60 million to Myonex upon execution of the warrant to purchase common stock agreement in May 2018;
- \$5.6 million decrease in collaboration cost sharing with Summit as it is winding down activities on its Utrophin platform; and
- \$5.9 million increase in research and other primarily driven by an increase in lab supplies as a result of an increase in headcount as well as sponsored research with academic institutions.

Non-GAAP research and development expenses were \$87.5 million and \$57.0 million for the second quarter of 2019 and 2018, respectively, an increase of \$30.6 million. Non-GAAP research and development expenses were \$168.9 million and \$100.3 million for the six months ended June 30, 2019 and 2018, respectively.

*Selling, general and administration*

Selling general and administrative expenses were \$67.4 million for the second quarter of 2019, compared to \$47.2 million for the same period of 2018, an increase of \$20.2 million. The increase in selling, general and administrative expenses primarily reflects the following:

- \$8.9 million increase in compensation and other personnel expenses primarily due to an increase in headcount;
- \$3.9 million increase in professional services primarily due to continuing global expansion;
- \$3.8 million increase in facility- and technology-related expense primarily due to continuing global expansion offset by a decrease in technology expense due to a change in allocation methodology;
- \$2.6 million increase in stock-based compensation primarily due to increases in headcount and stock price; and
- \$2.2 million decrease in restructuring credits due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility recorded during the second quarter of 2018.

Selling general and administrative expenses were \$128.0 million for the six months ended June 30, 2019, compared to \$90.5 million for the same period of 2018, an increase of \$37.5 million. The increase in selling, general and administrative expenses primarily reflects the following:

- \$19.3 million increase in compensation and other personnel expenses primarily due to an increase in headcount;
- \$5.3 million increase in facility- and technology-related expense primarily due to continuing global expansion offset by a decrease in technology expense due to a change in allocation methodology;
- \$5.2 million increase in stock-based compensation primarily due to increases in headcount and stock price;
- \$4.6 million increase in professional services primarily due to continuing global expansion; and
- \$2.2 million decrease in restructuring credits due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility recorded during the second quarter of 2018.

Non-GAAP selling, general and administrative expenses were \$52.3 million and \$37.3 million for the second quarter of 2019 and 2018, respectively, an increase of \$14.9 million. Non-GAAP selling, general and administrative expenses were \$100.1 million and \$71.0 million for the six months ended June 30, 2019 and 2018, respectively, an increase of \$29.1 million.

#### Acquired in-process research and development

As a result of the Myonexus acquisition, we recorded acquired in-process research and development expense of approximately \$173.2 million during the second quarter of 2019. There was no such transaction during the same period of 2018.

#### Amortization of in-licensed rights

For the three and six months ended June 30, 2019, and 2018, we recorded amortization of in-licensed rights of approximately \$0.2 million and \$0.4 million, respectively.

#### **Other loss**

#### Other expense, net

For the three and six months ended June 30, 2019, other expense, net was approximately \$0.9 million and \$1.0 million, respectively. For the three and six months ended June 30, 2018, other expense, net was approximately \$5.2 million and \$9.7 million, respectively. The decrease primarily reflected increases in interest income from higher balances of cash, cash equivalents and investments and amortization of investment discount as a result of an increase in interest rates.

#### ***Cash, Cash Equivalents, Investments and Restricted Cash and Investments***

The Company had approximately \$1.1 billion in cash, cash equivalents and investments as of June 30, 2019 compared to \$1.2 billion as of December 31, 2018. The decrease is primarily driven by the proceeds of the public offering of common stock in March 2019 offset by cash used to fund the Company's ongoing operations during the first half of 2019.

### *Use of Non-GAAP Measures*

In addition to the GAAP financial measures set forth in this press release, the Company has included certain non-GAAP measurements. The non-GAAP loss is defined by the Company as GAAP net loss excluding interest expense/(income), income tax expense/(benefit), depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP research and development expenses are defined by the Company as GAAP research and development expenses excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP selling, general and administrative expenses are defined by the Company as GAAP selling, general and administrative expenses excluding depreciation and amortization expense, stock-based compensation expense and other items.

#### 1. Interest, tax, depreciation and amortization

Interest income and expense amounts can vary substantially from period to period due to changes in cash and debt balances and interest rates driven by market conditions outside of the Company's operations. Tax amounts can vary substantially from period to period due to tax adjustments that are not directly related to underlying operating performance. Depreciation expense can vary substantially from period to period as the purchases of property and equipment may vary significantly from period to period and without any direct correlation to the Company's operating performance. Amortization expense associated with in-licensed rights as well as patent costs are amortized over a period of several years after acquisition or patent application or renewal and generally cannot be changed or influenced by management.

#### 2. Stock-based compensation expenses

Stock-based compensation expenses represent non-cash charges related to equity awards granted by Sarepta. Although these are recurring charges to operations, management believes the measurement of these amounts can vary substantially from period to period and depend significantly on factors that are not a direct consequence of operating performance that is within management's control. Therefore, management believes that excluding these charges facilitates comparisons of the Company's operational performance in different periods.

#### 3. Other items

The Company evaluates other items of expense and income on an individual basis. It takes into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relate to the Company's ongoing business operations, and (c) whether the Company expects the items to continue on a regular basis. These other items include up-front and milestone payments and acquired in-process research and development expense. The Company excludes up-front, milestone, and acquired in-process research and development expenses associated with its license and collaboration agreements from its financial results and research and development expenses because the Company does not consider them to be normal operating expenses due to their nature, variability of amounts, and lack of predictability as to occurrence and/or timing. Up-front payments are made at the commencement of a collaborative relationship or a license agreement anticipated to continue for a multi-year period and provide the Company with intellectual property rights, option rights and other rights with respect to particular programs. Milestone payments are made when certain development, regulatory and sales milestone events are achieved. The variability of amounts and lack of predictability of collaboration- and license-related up-front and milestone payment makes the identification of trends in the Company's ongoing research and development activities more difficult. As a result of the Myonex acquisition, the Company recorded acquired in-process research and development expense, which represents a non-recurring expense and, therefore, was treated as a non-GAAP adjustment item. The Company believes the presentation of adjusted research and development, which does not include license- and collaboration-related up-front and milestone expenses, provides useful and meaningful information about its ongoing research and development activities by enhancing investors' understanding of the Company's normal, recurring operating research and development expenses and facilitates comparisons between periods and with respect to projected performance.

The Company uses these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. The Company also believes these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating the Company's performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or to replace the presentation of the Company's financial results in accordance with GAAP. Use of the terms non-GAAP research and development expenses, non-GAAP selling, general and administrative expenses, non-GAAP other income and loss adjustments, non-GAAP income tax expense, non-GAAP net loss, and non-GAAP basic and diluted net loss per share may differ from similar measures reported by other companies, which may limit comparability, and are not based on any comprehensive set of accounting rules or principles. All relevant non-GAAP measures are reconciled from their respective GAAP measures in the attached table "Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures."



## **About EXONDYS 51**

EXONDYS 51 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

## **Important Safety Information About EXONDYS 51**

Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

In the 88 patients who received  $\geq 30$  mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in  $\geq 10\%$  of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

For further information, please see the full Prescribing Information.

## **About Sarepta Therapeutics**

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for 6 Limb-girdle

muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit [www.sarepta.com](http://www.sarepta.com).

## **Forward-Looking Statements**

*In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to the potential benefits of our product, product candidates and programs, including those with strategic partners; an expected FDA PDUFA date for golodirsen of August 19, 2019; a target PDUFA date for casimersen in the first half of 2020; our gene therapy position in 2019 and our plans to perform with the same level of attention to detail and urgency; PPMO's potential to increase drug concentration in muscle tissue; and our mission to profoundly improve and extend the lives of patients with rare genetic-based diseases.*

*These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: we may not be able to meet expectations with respect to EXONDYS 51 sales or attain the net revenues we anticipate for 2019, profitability or positive cash-flow from operations; we may not be able to comply with all FDA post-approval commitments and requirements with respect to EXONDYS 51 in a timely manner or at all; the expected benefits and opportunities related to our programs and collaborations may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; Sarepta's dependence on certain manufacturers to produce its product candidates, including any inability on Sarepta's part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of product to successfully support various programs; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results; our data for golodirsen, casimersen, SRP-9001, the LGMD programs and/or other programs may not be sufficient for obtaining regulatory approval;*

*if the actual number of patients suffering from the diseases we aim to treat is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; Sarepta may not be able to execute on its business plans, including meeting its expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing its product candidates to market, for various reasons, some of which may be outside of Sarepta's control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover Sarepta's product candidates; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2018 and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.*

*Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.*

#### **Internet Posting of Information**

*We routinely post information that may be important to investors in the 'For Investors' section of our website at [www.sarepta.com](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.*

Sarepta Therapeutics, Inc.  
Condensed Consolidated Statements of Operations  
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2019	2018	2019	2018
Revenues:				
Product, net	\$ 94,668	\$ 73,529	\$ 181,679	\$ 138,133
Total revenues	<u>94,668</u>	<u>73,529</u>	<u>181,679</u>	<u>138,133</u>
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	15,919	6,735	27,982	12,317
Research and development	113,266	122,848	203,819	169,052
Selling, general and administrative	67,393	47,156	127,959	90,497
Acquired in-process research and development	173,240	—	173,240	—
Amortization of in-licensed rights	217	217	433	433
Total cost and expenses	<u>370,035</u>	<u>176,956</u>	<u>533,433</u>	<u>272,299</u>
Operating loss	<u>(275,367)</u>	<u>(103,427)</u>	<u>(351,754)</u>	<u>(134,166)</u>
Other loss:				
Other expense, net	<u>(862)</u>	<u>(5,218)</u>	<u>(1,034)</u>	<u>(9,703)</u>
Other loss	<u>(862)</u>	<u>(5,218)</u>	<u>(1,034)</u>	<u>(9,703)</u>
Loss before income tax expense	(276,229)	(108,645)	(352,788)	(143,869)
Income tax expense	<u>174</u>	<u>622</u>	<u>258</u>	<u>761</u>
Net loss	<u>(276,403)</u>	<u>(109,267)</u>	<u>(353,046)</u>	<u>(144,630)</u>
Net loss per share - basic and diluted	\$ (3.74)	\$ (1.67)	\$ (4.85)	\$ (2.22)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	73,958	65,484	72,850	65,060

Sarepta Therapeutics, Inc.  
Reconciliation of GAAP Financial Measures to Non-GAAP Financial Measures  
(unaudited, in thousands, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
GAAP net loss	\$ (276,403)	\$ (109,267)	\$ (353,046)	\$(144,630)
Interest expense, net	741	4,689	1,383	9,192
Income tax expense	174	622	258	761
Depreciation and amortization expense	6,233	2,873	11,113	5,125
Stock-based compensation expense	19,762	15,279	35,901	25,805
Restructuring expense	—	(2,222)	—	(2,222)
Up-front, milestone, and other expenses	15,078	60,000	16,200	60,000
Acquired in-process research and development	173,240	—	173,240	—
Non-GAAP net loss	<u>\$ (61,175)</u>	<u>\$ (28,026)</u>	<u>\$ (114,951)</u>	<u>\$ (45,969)</u>

Non-GAAP net loss per share:

Basic and diluted	\$ (0.83)	\$ (0.43)	\$ (1.58)	\$ (0.71)
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Weighted average number of shares of common stock outstanding for computing:

Basic and diluted	73,958	65,484	72,850	65,060
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	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
GAAP research and development expenses	\$ 113,266	\$ 122,848	\$ 203,819	\$ 169,052
Up-front, milestone, and other expenses	(15,078)	(60,000)	(16,200)	(60,000)
Stock-based compensation expense	(6,923)	(5,029)	(12,010)	(7,089)
Depreciation and amortization expense	(3,725)	(853)	(6,687)	(1,701)
Non-GAAP research and development expenses	<u>\$ 87,540</u>	<u>\$ 56,966</u>	<u>\$ 168,922</u>	<u>\$ 100,262</u>

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
GAAP selling, general and administrative expenses	\$ 67,393	\$ 47,156	\$ 127,959	\$ 90,497
Stock-based compensation expense	(12,839)	(10,250)	(23,891)	(18,716)
Depreciation and amortization expense	(2,291)	(1,803)	(3,993)	(2,991)
Restructuring expense	—	2,222	—	2,222
Non-GAAP selling, general and administrative expenses	<u>\$ 52,263</u>	<u>\$ 37,325</u>	<u>\$ 100,075</u>	<u>\$ 71,012</u>

Sarepta Therapeutics, Inc.  
Condensed Consolidated Balance Sheets  
(unaudited, in thousands, except share and per share data)

	As of June 30, 2019	As of December 31, 2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 808,591	\$ 370,829
Short-term investments	294,478	803,083
Accounts receivable	56,981	49,044
Inventory	156,569	125,445
Other current assets	110,799	77,782
Total current assets	1,427,418	1,426,183
Property and equipment, net of accumulated depreciation of \$38,398 and \$28,149 as of June 30, 2019, and December 31, 2018, respectively	117,201	97,024
Intangible assets, net of accumulated amortization of \$4,685 and \$3,852 as of June 30, 2019, and December 31, 2018, respectively	11,825	11,574
Right of use asset, net	39,449	—
Other assets	151,860	107,294
Total assets	\$ 1,747,753	\$ 1,642,075
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 44,045	\$ 33,829
Accrued expenses	107,328	134,095
Deferred revenue	3,303	3,303
Other current liabilities	7,413	2,463
Total current liabilities	162,089	173,690
Long-term debt	431,040	420,554
Lease liabilities	50,209	—
Deferred rent and other	5,248	15,555
Total liabilities	648,586	609,799
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, \$0.0001 par value, 99,000,000 shares authorized; 74,327,767 and 71,071,887 issued and outstanding at June 30, 2019, and December 31, 2018, respectively	7	7
Additional paid-in capital	3,031,050	2,611,294
Accumulated other comprehensive income (loss)	82	(99)
Accumulated deficit	(1,931,972)	(1,578,926)
Total stockholders' equity	1,099,167	1,032,276
Total liabilities and stockholders' equity	\$ 1,747,753	\$ 1,642,075

*Note: As of January 1, 2019, the Company adopted the requirements of Accounting Standards Codification 842, Leases, using the modified retrospective method as of the effective date, and as a result, Other Liabilities are not comparable to the prior periods presented.*

Source: Sarepta Therapeutics, Inc.

Sarepta Therapeutics, Inc.

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