



Sarepta Therapeutics Announces Third Quarter 2019 Financial Results and Recent Corporate Developments

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

“EXONDYS 51® (eteplirsen) enjoyed another strong quarter, with third quarter 2019 revenues of \$99.0 million, a 26% increase over the same quarter in 2018. Based on current performance, we have raised our full-year 2019 guidance from a range of \$365 to \$375 million to \$370 to \$380 million,” stated Doug Ingram, Sarepta’s President and CEO.

Mr. Ingram continued, “We have also made significant progress advancing our gene therapy platform. In the quarter, we increased the study enrollment size of our current double-blind, placebo-controlled trial, significantly increasing study power. We are now actively screening and dosing patients at two sites, with the goal of completing all dosing by year end. We have also made progress on gene therapy manufacturing capacity, analytical development and process development and remain on track to initiate our next trial, using commercial process supply, by mid-2020. We also announced positive nine-month functional results from a cohort of three limb-girdle muscular dystrophy Type 2E clinical trial participants who received our investigational gene therapy candidate, SRP-9003. As reported at the World Muscle Conference in Copenhagen on October 5th, at Day 270 all three participants showed improvements from baseline across all functional measures, including the modified NSAA, time to rise, four-stair climb, 100-meter walk test and 10-meter walk test, and no new safety signals were observed.”

Mr. Ingram concluded, “While not without its challenges, 2019 has been one marked by a significant advancement of our platform. We continued to exceed revenue expectations, built our pipeline and advanced our programs, reported additional positive and validating clinical data, advanced our manufacturing strategy, and expanded and enhanced the expertise of our team of Sarepta employees. Through these efforts we have taken great strides toward our goal to be among the world’s leaders in genetic medicine for those with life-limiting and too often life-ending rare diseases.”

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 8998299. Please specify to the operator that you would like to join the "Sarepta Third Quarter 2019 Earnings Call." The conference call will be webcast live under the investor relations section of Sarepta's website at 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 \$126.3 million and \$76.4 million, or \$1.70 and \$1.15 per basic and diluted share for the third quarter of 2019 and 2018, respectively. On a non-GAAP basis, the net loss for the third quarter of 2019 was \$84.4 million, or \$1.14 per basic and diluted share, compared to a net loss of \$37.1 million, or \$0.56 per basic and diluted share for the same period of 2018.

On a GAAP basis, for the nine months ended September 30, 2019, the Company reported a net loss of \$479.4 million, or \$6.54 per basic and diluted share, compared to a net loss of \$221.0 million, or \$3.38 per basic and diluted share for the same period of 2018. On a non-GAAP basis, the net loss for the nine months ended September 30, 2019 was \$199.4 million, or \$2.72 per basic and diluted share, compared to a net loss of \$83.1 million, or \$1.27 per basic and diluted share for the same period of 2018.

Net Revenues

For the three months ended September 30, 2019, the Company recorded net revenues of \$99.0 million, compared to net revenues of \$78.5 million for the same period of 2018, an increase of \$20.5 million. For the nine months ended September 30, 2019, the Company recorded net revenues of \$280.7 million, compared to net revenues of \$216.6 million for nine months ended September 30, 2018, an increase of \$64.1 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 September 30, 2019, cost of sales (excluding amortization of in-licensed rights) was \$13.0 million, compared to \$8.7 million for the same period of 2018. For the nine months

ended September 30, 2019, cost of sales (excluding amortization of in-licensed rights) was \$41.0 million, compared to \$21.1 million for the same period of 2018. The increases primarily reflect 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. 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 nine months ended September 30, 2018 compared with the same period of 2019.

Research and development

Research and development expenses were \$133.9 million for the third quarter of 2019, compared to \$86.6 million for the same period of 2018, an increase of \$47.3 million. The increase in research and development expenses primarily reflects the following:

- \$35.8 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of the Company's micro-dystrophin program, the Company's ESSENCE program and initiation of certain post-market studies for EXONDYS 51. The increases were offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- \$7.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- \$7.3 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts as well as a change in methodology in allocation of technology expense;
- \$3.7 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- \$3.0 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of the Company's research and development pipeline;
- \$2.5 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;
- \$5.9 million decrease in up-front, milestone, and other expenses primarily due to \$10.0 million of an up-front payment due as a result of execution of a license agreement during the third quarter of 2019, as compared with a \$10.0 million milestone payment to Myonex Therapeutics, Inc. ("Myonex") as a result of the achievement of a development milestone in September 2018 as well as \$8.0 million related to the purchase of a license to develop, manufacture and

commercialize a pre-clinical Pompe product candidate under the license agreement with Lacerta Therapeutics, Inc. (“Lacerta”) in August 2018;

- \$5.3 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in the Company’s PPMO platform; and
- \$1.6 million decrease in collaboration cost sharing with Summit (Oxford) Ltd. (“Summit”) as it is winding down activities on its Utrophin platform.

Research and development expenses were \$337.8 million for the nine months ended September 30, 2019, compared to \$255.6 million for the same period of 2018, an increase of \$82.2 million. The increase in research and development expenses primarily reflects the following:

- \$71.1 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of the Company’s micro-dystrophin program, the Company’s ESSENCE program and initiation of certain post-market studies for EXONDYS 51. The increases were offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- \$29.1 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- \$22.9 million increase in facility- and technology-related expenses due to the Company’s continuing expansion efforts as well as a change in methodology in allocation of technology expense;
- \$8.6 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- \$8.5 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;
- \$4.2 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of the Company’s research and development pipeline;
- \$49.7 million decrease in up-front, milestone, and other expenses primarily due to \$25.5 million of up-front payments as a result of license agreements executed during the nine months ended September 30, 2019, as compared with an up-front payment of \$60.0 million to Myonex upon execution of the warrant to purchase common stock agreement in May 2018, a milestone payment of \$10.0 million to Myonex upon achievement of a development milestone in September 2018 and \$8.0 million related to the purchase of a license to develop, manufacture

and commercialize a pre-clinical Pompe product candidate under the license agreement with Lacerta in August 2018;

- \$7.2 million decrease in collaboration cost sharing with Summit as it is winding down activities on its Utrophin platform; and
- \$5.4 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in the Company's PPMO platform.

Non-GAAP research and development expenses were \$110.5 million and \$64.2 million for the third quarter of 2019 and 2018, respectively, an increase of \$46.3 million. Non-GAAP research and development expenses were \$279.4 million and \$164.5 million for the nine months ended September 30, 2019 and 2018, respectively, an increase of \$114.9 million.

Selling, general and administration

Selling general and administrative expenses were \$75.4 million for the third quarter of 2019, compared to \$53.0 million for the same period of 2018, an increase of \$22.4 million. The increase in selling, general and administrative expenses primarily reflects the following:

- \$8.4 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- \$5.4 million increase in stock-based compensation primarily due to increases in headcount and stock price;
- \$5.2 million increase in professional services primarily due to continuing global expansion; and
- \$4.3 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.

Selling, general and administrative expenses were \$203.4 million for the nine months ended September 30, 2019, compared to \$143.5 million for the same period of 2018, an increase of \$59.9 million. The increase in selling, general and administrative expenses primarily reflects the following:

- \$27.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- \$10.6 million increase in stock-based compensation primarily due to increases in headcount and stock price;
- \$9.7 million increase in professional services primarily due to continuing global expansion;

- \$7.9 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; 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 the Company's Corvallis facility recorded during the second quarter of 2018.

Non-GAAP selling, general and administrative expenses were \$59.6 million and \$42.5 million for the third quarter of 2019 and 2018, respectively, an increase of \$17.1 million. Non-GAAP selling, general and administrative expenses were \$159.7 million and \$113.5 million for the nine months ended September 30, 2019 and 2018, respectively, an increase of \$46.2 million.

Acquired in-process research and development

As a result of the Myonexus acquisition, the Company 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 both the three and nine months ended September 30, 2019, and 2018, the Company recorded amortization of in-licensed rights of approximately \$0.2 million and \$0.6 million, respectively.

Other expense, net

For the three and nine months ended September 30, 2019, other expense, net was approximately \$2.5 million and \$3.5 million, respectively. For the three and nine months ended September 30, 2018, other expense, net was approximately \$7.0 million and \$16.7 million, respectively. The decrease primarily reflected a decrease in term loan termination expense and an increase in 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 September 30, 2019 compared to \$1.2 billion as of December 31, 2018. The decrease is primarily driven by cash used to fund the Company's ongoing operations during the first three quarters of 2019 offset by the proceeds of the public offering of common stock in March 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 ≥ 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 Limb-girdle muscular dystrophy diseases (LGMD), 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.

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 our full-year 2019 guidance of \$370 to \$380 million; our goal of completing all dosing in our current double-blind, placebo-controlled trial by year end; our plan to initiate our next micro-dystrophin gene therapy trial, using commercial process supply, by mid-2020; our goal to be among the world's leaders in genetic medicine for those with life-limiting and ending rare diseases; 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; 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 early results from a clinical trial do not necessarily predict final results; our data for golodirsen, casimersen, SRP-9001, SRP-9003 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. 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 September 30, | | For the Nine Months Ended September 30, | |
|--|---|--------------------|--|---------------------|
| | 2019 | 2018 | 2019 | 2018 |
| Revenues: | | | | |
| Product, net | \$ 99,041 | \$ 78,486 | \$ 280,720 | \$ 216,619 |
| Total revenues | <u>99,041</u> | <u>78,486</u> | <u>280,720</u> | <u>216,619</u> |
| Cost and expenses: | | | | |
| Cost of sales (excluding amortization of in-licensed rights) | 13,037 | 8,741 | 41,019 | 21,058 |
| Research and development | 133,949 | 86,584 | 337,768 | 255,636 |
| Selling, general and administrative | 75,429 | 53,044 | 203,388 | 143,541 |
| Acquired in-process research and development | — | — | 173,240 | — |
| Amortization of in-licensed rights | 216 | 216 | 649 | 649 |
| Total cost and expenses | <u>222,631</u> | <u>148,585</u> | <u>756,064</u> | <u>420,884</u> |
| Operating loss | <u>(123,590)</u> | <u>(70,099)</u> | <u>(475,344)</u> | <u>(204,265)</u> |
| Other loss: | | | | |
| Other expense, net | <u>(2,510)</u> | <u>(6,968)</u> | <u>(3,544)</u> | <u>(16,671)</u> |
| Other loss | <u>(2,510)</u> | <u>(6,968)</u> | <u>(3,544)</u> | <u>(16,671)</u> |
| Loss before income tax expense | (126,100) | (77,067) | (478,888) | (220,936) |
| Income tax expense (benefit) | 226 | (674) | 484 | 87 |
| Net loss | <u>\$ (126,326)</u> | <u>\$ (76,393)</u> | <u>\$ (479,372)</u> | <u>\$ (221,023)</u> |
| Net loss per share - basic and diluted | \$ (1.70) | \$ (1.15) | \$ (6.54) | \$ (3.38) |
| Weighted average number of shares of common stock used in computing basic and diluted net loss per share | 74,177 | 66,209 | 73,298 | 65,454 |

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|-------------------------------------|--------------------|------------------------------------|--------------------|
| | 2019 | 2018 | 2019 | 2018 |
| GAAP net loss | \$ (126,326) | \$ (76,393) | \$ (479,372) | \$ (221,023) |
| Interest expense, net | 2,136 | 6,909 | 3,519 | 16,101 |
| Income tax expense (benefit) | 226 | (674) | 484 | 87 |
| Depreciation and amortization expense | 6,741 | 3,593 | 17,854 | 8,718 |
| Stock-based compensation expense | 20,637 | 11,484 | 56,538 | 37,289 |
| Restructuring benefit | — | — | — | (2,222) |
| Up-front, milestone, and other expenses | 12,146 | 18,000 | 28,346 | 78,000 |
| Acquired in-process research and development | — | — | 173,240 | — |
| Non-GAAP net loss | <u>\$ (84,440)</u> | <u>\$ (37,081)</u> | <u>\$ (199,391)</u> | <u>\$ (83,050)</u> |
| Non-GAAP net loss per share: | | | | |
| Basic and diluted | \$ (1.14) | \$ (0.56) | \$ (2.72) | \$ (1.27) |
| Weighted average number of shares of common stock outstanding for computing: | | | | |
| Basic and diluted | 74,177 | 66,209 | 73,298 | 65,454 |
| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
| | 2019 | 2018 | 2019 | 2018 |
| GAAP research and development expenses | \$ 133,949 | \$ 86,584 | \$ 337,768 | \$ 255,636 |
| Up-front, milestone, and other expenses | (12,146) | (18,000) | (28,346) | (78,000) |
| Stock-based compensation expense | (6,972) | (3,260) | (18,982) | (10,349) |
| Depreciation and amortization expense | (4,365) | (1,092) | (11,052) | (2,793) |
| Non-GAAP research and development expenses | <u>\$ 110,466</u> | <u>\$ 64,232</u> | <u>\$ 279,388</u> | <u>\$ 164,494</u> |
| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
| | 2019 | 2018 | 2019 | 2018 |
| GAAP selling, general and administrative expenses | \$ 75,429 | \$ 53,044 | \$ 203,388 | \$ 143,541 |
| Stock-based compensation expense | (13,665) | (8,224) | (37,556) | (26,940) |
| Depreciation and amortization expense | (2,160) | (2,285) | (6,153) | (5,276) |
| Restructuring benefit | — | — | — | 2,222 |
| Non-GAAP selling, general and administrative expenses | <u>\$ 59,604</u> | <u>\$ 42,535</u> | <u>\$ 159,679</u> | <u>\$ 113,547</u> |

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

| | As of September 30, 2019 | As of December 31, 2018 |
|---|--------------------------------|-------------------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 724,829 | \$ 370,829 |
| Short-term investments | 324,063 | 803,083 |
| Accounts receivable | 68,032 | 49,044 |
| Inventory | 166,360 | 125,445 |
| Other current assets | 79,015 | 77,782 |
| Total current assets | 1,362,299 | 1,426,183 |
| Property and equipment, net of accumulated depreciation of \$44,701 and \$28,149 as of September 30, 2019, and December 31, 2018, respectively | 119,532 | 97,024 |
| Intangible assets, net of accumulated amortization of \$5,091 and \$3,852 as of September 30, 2019, and December 31, 2018, respectively | 11,975 | 11,574 |
| Right of use asset, net | 39,493 | — |
| Other assets | 169,171 | 107,294 |
| Total assets | \$ 1,702,470 | \$ 1,642,075 |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | \$ 81,946 | \$ 33,829 |
| Accrued expenses | 121,663 | 134,095 |
| Deferred revenue | 3,303 | 3,303 |
| Other current liabilities | 8,944 | 2,463 |
| Total current liabilities | 215,856 | 173,690 |
| Long-term debt | 436,421 | 420,554 |
| Lease liabilities | 49,741 | — |
| Deferred rent and other | 5,248 | 15,555 |
| Total liabilities | 707,266 | 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; 74,504,835 and 71,071,887 issued and outstanding at September 30, 2019, and December 31, 2018, respectively | 7 | 7 |
| Additional paid-in capital | 3,053,420 | 2,611,294 |
| Accumulated other comprehensive income (loss) | 75 | (99) |
| Accumulated deficit | (2,058,298) | (1,578,926) |
| Total stockholders' equity | 995,204 | 1,032,276 |
| Total liabilities and stockholders' equity | \$ 1,702,470 | \$ 1,642,075 |

(1) 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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