



## Sarepta Therapeutics Announces First Quarter 2020 Financial Results and Recent Corporate Developments

5/6/20

– Net sales of \$100.4 million, a 15% increase over same quarter of prior year –

– \$2.2 billion of cash on hand to drive clinical programs without distraction from COVID-19 pandemic –

CAMBRIDGE, Mass., May 06, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the three months ended March 31, 2020.

"I am proud to report that with our typical sense of urgency the Sarepta team rapidly adapted to the challenges and obstacles posed by the COVID-19 pandemic, assessing and minimizing potential impacts, working to protect our patients, protecting our facility-dependent workers, and driving our mission forward. In mid-March, we smoothly transitioned the majority of our employees to work-from-home status and, for the approximately 10% of our workforce that is facility-dependent, we imposed thoughtful protocols designed to keep them safe and comfortable as they come into the labs and facilities to keep our experiments, CMC work and other facility-focused work moving without interruption," stated Doug Ingram, Sarepta's president and chief executive officer. "In addition to serving the patient community with our therapies we have made donations to patient advocacy organizations that are providing direct relief to impacted patients and caregivers. We have contributed on a local level by donating personal protective equipment to area hospitals and by donating to the Boston Resiliency Fund. And as we have reported, we have designed and manufactured a number of RNA-based candidates as potential COVID-19 therapies that are being tested by the Department of Defense now as potential therapies for this disease."

Mr. Ingram continued, "I am pleased to report that we achieved net sales of \$100.4 million in the first quarter, a 15% increase over the same quarter last year. Importantly in light of the external environment, we also closed the first quarter with \$2.2 billion of cash on hand and are fortunate to be in a privileged position to weather this crisis, remain focused on patients and our mission, and come through this challenging period strongly. We continue to provide an uninterrupted supply of our commercial therapies, EXONDYS 51 and VYONDYS 53, to serve the Duchenne community. On the clinical front, our placebo-controlled gene therapy clinical trial, study 102, for SRP-9001 is on track to read out in the first quarter of 2021 as anticipated. Additionally, our process development, assay and manufacturing work for SRP-9001 is progressing and we continue to expect GMP material to be ready in July. While the pandemic is causing short term challenges and obstacles to address, our strategy remains unchanged and we remain on mission."

### Impact of COVID-19 Pandemic:

#### • Business Operations:

- The Company has activated business continuity plans to minimize disruption to patients. There has been minimal disruption to the Company's commercial and clinical supply chains and Sarepta continues to provide an uninterrupted supply of its approved or investigational therapies to patients.
- Sarepta is operating under guidance from federal agencies, including the US Food and Drug Administration (FDA) and Centers for Disease Control and Prevention (CDC), which designate healthcare companies as "critical infrastructure" with a special responsibility to maintain normal work schedules. Importantly, our partners and suppliers share these special responsibilities to maintain operations as critical infrastructure and ensure the integrity of the supply chain.
- Facility-dependent employees, including those needed to maintain manufacturing and clinical research, are reporting to work under strict protocols designed to ensure they remain healthy.
- The company is supporting employees, including remote work, and leveraging virtual meeting technology and encouraging them to follow local guidance.

#### • Clinical Operations:

- For clinical trials, Sarepta is informed by the direction and flexibility provided by the United States Food and Drug Administration "[FDA guidance on clinical trial conduct during the pandemic](#)" and the Company is working closely with clinical trial sites to ensure that patient safety remains the top priority.
  - With respect to Sarepta's active gene therapy trial, study 102 for SRP-9001, all dosing needed for the 48-week analysis has taken place and we are working within the FDA Guidance to avoid or minimize delays in trial follow-up visits during this acute period.
  - For trials that involve weekly or monthly dosing, Sarepta is working to adapt protocols to allow for virtual interactions, while remaining fully compliant with Good Clinical Practice and continuing to advance these important investigational treatments.

### First Quarter 2020 and Recent Corporate Developments:

- On April 28, Sarepta announced it had entered into a Cooperative Research and Development Agreement (CRADA) with the United States Army Medical Research Institute of Infectious Diseases (USAMRIID), the Department of Defense's lead laboratory for medical biological defense research. The purpose of the CRADA is to jointly identify antisense oligonucleotides using Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) platform with activity against SARS-CoV-2 for the potential treatment of COVID-19.
- In March, Sarepta completed its sale of the rare pediatric disease Priority Review Voucher ("PRV") for net proceeds of \$108.1 million. The company received the PRV from the FDA in connection with the approval of VYONDYS 53.

### **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 2553748. Please specify to the operator that you would like to join the "Sarepta First Quarter 2020 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 \$17.5 million and \$76.6 million, or \$0.23 and \$1.07 per basic and diluted share for the first quarter of 2020 and 2019, respectively. On a non-GAAP basis, the net loss for the first quarter of 2020 was \$79.8 million, or \$1.04 per basic and diluted share, compared to a net loss of \$53.8 million, or \$0.75 per basic and diluted share for the same period of 2019.

### **Revenues**

For the three months ended March 31, 2020, the Company recorded net product revenues of \$100.4 million, compared to net revenues of \$87.0 million for the same period of 2019, an increase of \$13.4 million. The increase primarily reflects the continuing increase in demand for the Company's products in the U.S.

In the quarter ended March 31, 2020, the Company recognized \$13.2 million of collaboration revenue, which relates to the Company's collaboration arrangement with F. Hoffman-La Roche Ltd. ("Roche"). In February 2020, the Company received an aggregate of approximately \$1.2 billion in cash consideration from Roche, consisting of an up-front payment and an equity investment in the Company. Of that amount, \$342.7 million is being recognized as revenue on a straight-line basis over the performance period, estimated to be through the fourth quarter of 2023.

### **Cost and Operating Expenses**

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

For the first quarter of 2020, cost of sales (excluding amortization of in-licensed rights) was \$12.6 million, compared to \$12.1 million for the same period of 2019, an increase of \$0.5 million. 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 the Company's products. This was partially offset by write-offs of certain batches of EXONDYS 51 not meeting the Company's quality specifications for the three months ended March 31, 2019, with no similar activity for the three months ended March 31, 2020.

#### *Research and development*

Research and development expenses were \$136.1 million for the first quarter of 2020, compared to \$90.6 million for the same period of 2019, an increase of \$45.5 million. The increase in research and development expenses primarily reflects the following:

- \$43.3 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of the Company's micro-dystrophin program and the Company's ESSENCE program. The increases were offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- \$7.4 million increase in up-front, milestone and other expenses primarily due to \$8.8 million of milestone expenses accrued to an academic institution during the three months ended March 31, 2020;
- \$6.4 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- \$4.2 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- \$2.4 million increase in collaboration cost sharing with Genethon on its micro-dystrophin drug candidates and Lysogene S.A on its MPS IIIA drug candidates;
- \$2.1 million increase in facility- and technology-related expenses due to the Company's continuing global expansion efforts;
- \$1.0 million increase in research and other primarily driven by an increase in sponsored research with academic institutions;
- \$4.6 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in the Company's

PPMO platform; and

- \$16.4 million offset to expense associated with a collaboration reimbursement from Roche.

Non-GAAP research and development expenses were \$114.2 million and \$81.4 million for the first quarter of 2020 and 2019, respectively, an increase of \$32.8 million.

#### *Selling, general and administration*

Selling general and administrative expenses were \$82.8 million for the first quarter of 2020, compared to \$60.6 million for the same period of 2019, an increase of \$22.2 million. The increase in selling, general and administrative expenses primarily reflects the following:

- \$14.3 million increase in professional services primarily due to a transaction fee for the Roche transaction;
- \$3.7 million increase in stock-based compensation primarily due to increases in headcount and stock price;
- \$2.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount; and
- \$1.7 million increase in facility- and technology-related expense primarily due to continuing global expansion.

Non-GAAP selling, general and administrative expenses were \$54.5 million and \$47.8 million for the first quarter of 2020 and 2019, respectively, an increase of \$6.7 million.

#### *Amortization of in-licensed rights*

For both the three months ended March 31, 2020 and 2019, the Company recorded amortization of in-licensed rights of approximately \$0.2 million. This is related to the amortization of the in-licensed right assets recognized as a result of agreements the Company entered into with BioMarin and UWA upon the first commercial sale of EXYONDYS 51 and VYONDYS 53.

#### *Gain from Sale of Priority Review Voucher*

In February 2020, the Company entered into an agreement with Vifor (International) Ltd. to sell the rare pediatric disease Priority Review Voucher ("PRV") it received from the FDA in connection with the approval of VYONDYS 53. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in March 2020, the Company completed its sale of the PRV and received proceeds of \$108.1 million, net of commission, which was recorded as a gain from sale of the PRV as it did not have a carrying value at the time of the sale. There was no similar activity during the three months ended March 31, 2019.

#### *Other expense, net*

For the three months ended March 31, 2020 and 2019, other expense, net was approximately \$7.4 million and \$0.2 million, respectively. The increase primarily reflects the interest expense on the Company's debt facilities entered into in December 2019.

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

The Company had approximately \$2.2 billion in cash, cash equivalents and investments as of March 31, 2020 compared to \$1.1 billion as of December 31, 2019. The increase is primarily driven by the \$1.2 billion up-front payments received from the Roche collaboration and equity investment offset by cash used to fund the Company's ongoing operations during 2020.

#### **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 collaboration revenue and transaction cost related to the Roche transaction, up-front and milestone payments, acquired in-process research and development expense and gain from sale of PRV.

The Company excludes collaboration revenue and transaction cost associated with the Roche transaction from its GAAP results. While collaboration revenue is recurring, as the Company's ordinary activities do not include contracting with third parties to provide them with research and development services, collaboration revenue is treated as a non-GAAP item. Additionally, the transaction fee related to the Roche transaction is non-recurring and is excluded from its GAAP results.

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 sale of the PRV obtained as a result of the FDA approval of VYONDYS 53 in December 2019 is a non-recurring event and excluded from the Company's GAAP results.

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.

EXONDYS 51 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with EXONDYS 51. A clinical benefit of EXONDYS 51 has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

EXONDYS 51 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

#### **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 mg 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 VYONDYS 53**

VYONDYS 53 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

VYONDYS 53 is indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

VYONDYS 53 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

### **Important Safety Information for VYONDYS 53**

Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53-treated patients, some requiring treatment. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the VYONDYS 53 therapy.

Renal toxicity was observed in animals who received golodirsen. Although renal toxicity was not observed in the clinical studies with VYONDYS 53, renal toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Renal function should be monitored in patients taking VYONDYS 53. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of renal function in DMD patients. Measurement of glomerular filtration rate (GFR) by 24-hour urine collection prior to initiation of therapy is recommended. Monthly monitoring for proteinuria by dipstick urinalysis and monitoring of serum cystatin C every three months is recommended. In the case of a confirmed dipstick proteinuria of 2+ or greater or elevated serum cystatin C, a 24-hour urine collection to quantify proteinuria and assess GFR should be performed.

Adverse reactions observed in at least 20% of treated patients and greater than placebo were (VYONDYS 53, placebo): headache (41%, 10%), pyrexia (41%, 14%), fall (29%, 19%), abdominal pain (27%, 10%), nasopharyngitis (27%, 14%), cough (27%, 19%), vomiting (27%, 19%), and nausea (20%, 10%).

Other adverse reactions that occurred at a frequency greater than 5% of VYONDYS 53-treated patients and at a greater frequency than placebo were: administration site pain, back pain, pain, diarrhea, dizziness, ligament sprain, contusion, influenza, oropharyngeal pain, rhinitis, skin abrasion, ear infection, seasonal allergy, tachycardia, catheter site related reaction, constipation, and fracture.

For further information, please see the full [Prescribing Information](#).

### **About Sarepta Therapeutics**

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit [www.sarepta.com](http://www.sarepta.com) or follow us on [Twitter](#), [LinkedIn](#), [Instagram](#) and [Facebook](#).

### **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 financial position; the potential of our RNA-based candidates to treat COVID-19; the expected or potential impact of COVID-19 on our commercialization, clinical trials, manufacturing and other business operations; and expected plans and milestones, including the expectation to have a read out of study 102 for SRP-9001 in the first quarter of 2021 and the expectation that GMP material for SRP-9001 will be ready in July 2020.*

*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: the COVID-19 pandemic is expected to reduce our revenue and may negatively impact our ongoing and planned clinical trials, manufacturing and other business operations; we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; our dependence on certain manufacturers to produce our products and product candidates, including any inability on our 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; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, some of which may be outside of our 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 our product candidates; and those risks identified under the heading "Risk Factors" in our most recent Annual Report on Form 10-K for the year ended December 31, 2019 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)

	<b>For the Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
Revenues:		
Products, net	\$ 100,448	\$ 87,011
Collaboration	13,226	—
Total revenues	<u>113,674</u>	<u>87,011</u>
Cost and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	12,622	12,063
Research and development	136,144	90,553
Selling, general and administrative	82,768	60,566
Amortization of in-licensed rights	166	216
Total cost and expenses	<u>231,700</u>	<u>163,398</u>
Operating loss	<u>(118,026)</u>	<u>(76,387)</u>
Other income (loss):		
Gain from sale of Priority Review Voucher	108,069	—
Other expense, net	(7,420)	(172)
Total other income (loss)	<u>100,649</u>	<u>(172)</u>
Loss before income tax expense	(17,377)	(76,559)
Income tax expense	115	84
Net loss	<u>\$ (17,492)</u>	<u>\$ (76,643)</u>
Net loss per share - basic and diluted	\$ (0.23)	\$ (1.07)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	76,432	71,731

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

	<b>Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
GAAP net loss	\$ (17,492)	\$ (76,643)
Interest expense, net	8,512	642
Income tax expense	115	84
Gain from sale of Priority Review Voucher	(108,069)	—
Collaboration revenue	(13,226)	—
Depreciation and amortization expense	6,529	4,879
Stock-based compensation expense	24,024	16,139
Roche transaction costs	11,292	—
Up-front, milestone, and other expenses	8,533	1,122
Non-GAAP net loss	<u>\$ (79,782)</u>	<u>\$ (53,777)</u>

Non-GAAP net loss per share:		
Basic and diluted	\$ (1.04)	\$ (0.75)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	76,432	71,731

	<b>Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
GAAP research and development expenses	\$ 136,144	\$ 90,553
Up-front, milestone, and other expenses	(8,533)	(1,122)
Stock-based compensation expense	(9,249)	(5,087)
Depreciation and amortization expense	(4,177)	(2,961)
Non-GAAP research and development expenses	<u>\$ 114,185</u>	<u>\$ 81,383</u>

	<b>Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
GAAP selling, general and administrative expenses	\$ 82,768	\$ 60,566
Stock-based compensation expense	(14,775)	(11,052)
Depreciation and amortization expense	(2,186)	(1,702)
Roche transaction costs	(11,292)	—
Non-GAAP selling, general and administrative expenses	<u>\$ 54,515</u>	<u>\$ 47,812</u>

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

	<b>As of March 31, 2020</b>	<b>As of December 31, 2019</b>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 1,764,212	\$ 835,080
Short-term investments	406,940	289,668
Accounts receivable	106,875	90,879
Inventory	173,168	171,379
Other current assets	96,153	81,907
Total current assets	<u>2,547,348</u>	<u>1,468,913</u>
Property and equipment, net	137,325	129,620
Intangible assets, net	12,813	12,497
Right of use assets	63,097	37,933
Other non-current assets	186,805	173,859
Total assets	<u>\$ 2,947,388</u>	<u>\$ 1,822,822</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 26,595	\$ 68,094
Accrued expenses	176,298	185,527
Deferred revenue, current portion	91,073	3,303

Other current liabilities	12,463	7,843
Total current liabilities	<u>306,429</u>	<u>264,767</u>
Long-term debt	687,953	681,900
Lease liabilities	65,263	47,720
Deferred revenue, net of current portion	732,667	—
Other non-current liabilities	10,248	10,248
Total liabilities	<u>1,802,560</u>	<u>1,004,635</u>
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; 77,957,790 and 75,184,863 issued and outstanding at March 31, 2020, and December 31, 2019, respectively	8	8
Additional paid-in capital	3,455,689	3,112,130
Accumulated other comprehensive income, net of tax	624	50
Accumulated deficit	<u>(2,311,493)</u>	<u>(2,294,001)</u>
Total stockholders' equity	<u>1,144,828</u>	<u>818,187</u>
Total liabilities and stockholders' equity	<u>\$ 2,947,388</u>	<u>\$ 1,822,822</u>

Source: Sarepta Therapeutics, Inc.

Sarepta Therapeutics, Inc.

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