Sarepta Therapeutics Announces First Quarter 2024 Financial Results and Recent Corporate Developments

5/1/24

- ***Net product revenues for the first quarter 2024 totaled $359.5 million, a 55% increase over the same quarter of the prior year***
- ***ELEVIDYS net revenues for the quarter totaled $133.9 million***
- ***Achieved GAAP Earnings of $36.1 million for the first quarter 2024 and non-GAAP Earnings of $78.2 million for the same period***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 1, 2024-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported financial results for the first quarter 2024.

"We are pleased to announce another strong quarter of growth from our four approved therapies, posting net product revenue of $359.5 million, a 55% increase over the same quarter of the prior year, and achieving profitability on a GAAP and non-GAAP basis," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "In particular, our recently approved gene therapy, ELEVIDYS, achieved nearly $134.0 million in net product revenue in the quarter. Although its initial label is quite narrow, ELEVIDYS has posted cumulative sales of over $334.0 million since its approval in June of last year, far exceeding performance of all other gene therapies approved in the last few years combined. Working with the FDA, we continue to productively prosecute our BLA supplement to expand the ELEVIDYS addressable population, with a target action date of June 21, 2024. If successful, 2024 could be the most profound year yet in our fight against the effects of Duchenne muscular dystrophy and a bellwether for the transformative potential of gene therapy for rare disease."

First Quarter 2024 and Recent Developments:

- **U.S. FDA review of efficacy supplement to expand the ELEVIDYS indication is ongoing:** The Company is currently awaiting U.S. Food and Drug Administration (FDA) Priority Review of its efficacy supplement to the Biologics License Application (BLA) for ELEVIDYS (delandistrogene moxeparvovec-rokl) where the review goal date is June 21, 2024. At acceptance of the BLA in February, the Agency also confirmed they are not planning to hold an advisory committee to discuss the supplement. The goals of the efficacy supplement are twofold:
  - To expand the labeled indication for ELEVIDYS as follows: "[ELEVIDYS is indicated for] ***the treatment of Duchenne muscular dystrophy (DMD) patients with a confirmed mutation in the DMD gene***."
  - To convert the ELEVIDYS accelerated approval to a traditional approval.
- **Real world evidence published in *Muscle & Nerve* (March 2024) finds eteplirsen treatment associated with longer survival compared to natural history:** The publication details real-world evidence showing that treatment with eteplirsen resulted in statistically significant survival benefits compared to a controlled natural history comparator group of Duchenne patients. In the analysis the survival age of the eteplirsen treated patients (n=579) was 32.8 years, 5.4 years longer than the median survival age of 27.4 years in the Duchenne natural history studies (n=1,224). Additionally, earlier initiation of eteplirsen treatment and longer duration of treatment were associated with increases in the survival benefit.

Other findings from the analysis include:

- Overall, eteplirsen-treated patients had a 66% reduction in risk of death compared with natural history control group.
- Longer duration of treatment with eteplirsen was associated with greater survival benefit, with patients treated with eteplirsen for longer than four years demonstrating an 85% lower risk of death compared to patients who had less than two years of treatment with eteplirsen.
- Patients receiving eteplirsen treatment for more than four years survived 32.8 years (95% confidence interval), and patients receiving eteplirsen between two to four years survived 29.4 years (95% confidence interval).
- Patients treated with eteplirsen for less than two years had a median survival of 28.1 years, compared to natural history of 27.4 years.
- The median survival age has not yet been reached in patients taking eteplirsen for longer than two years.

- In patients aged 10 to 28 years, for whom deaths are most likely to be observed, eteplirsen treatment had a 42% lower risk of death than age-matched natural history patients.

These findings add to the large and growing body of real-world evidence generated for eteplirsen, where treatment is associated with delays in time to loss of ambulation, improvements in lung function and various quality of life measures when compared to standard of care or natural history controls.

Conference Call

The event will be webcast live under the investor relations section of Sarepta's website at **https://investorrelations.sarepta.com/events-presentations** and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using **this online form.** After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

Q1 2024 Financial Highlights[1]

	For the Three Months Ended March 31,			
	2024	2023	Change	Change
	(in millions, except for per share amounts)		$	%
Total Revenues	$ 413.5	$ 253.5	$ 160.0	63%
Operating income (loss):				
GAAP	$ 34.9	$ (138.1)	$ 173.0	NM*
Non-GAAP	$ 83.7	$ (85.7)	$ 169.4	NM*
Net income (loss):				
GAAP	$ 36.1	$ (516.8)	$ 552.9	NM*
Non-GAAP	$ 78.2	$ (87.7)	$ 165.9	NM*
Diluted earnings (loss) per share				
GAAP	$ 0.37	$ (5.86)	$ 6.23	NM*
Non-GAAP	$ 0.73	$ (0.99)	$ 1.72	NM*

*NM: not meaningful

[1] For an explanation of our use of non-GAAP financial measures, please refer to the "Use of Non-GAAP Financial Measures" section later in this press release and for a reconciliation of each non-GAAP financial measure to the most comparable GAAP measures, see the table at the end of this press release.

	As of March 31, 2024	As of December 31, 2023
	(in millions)	
Cash, cash equivalents, and investments	$ 1,440.3	$ 1,691.8

Revenues

Total revenues increased by $160.0 million for the three months ended March 31, 2024 compared to the same period of 2023. The increase primarily reflects the increasing demand for ELEVIDYS as well as the $48.0 million of collaboration revenue recognized related to F. Hoffman-La Roche Ltd.'s ("Roche") declined option to acquire the ex-US rights to a certain external, early-stage Duchenne development program. Additionally, for the three months ended March 31, 2024, we recognized $5.8 million of contract manufacturing collaboration revenue associated with commercial ELEVIDYS supply delivered to Roche, with no similar activity for the same period of 2023.

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

Cost of sales (excluding amortization of in-license rights) increased by $15.5 million for the three months ended March 31, 2024, compared with the same period of 2023, which primarily reflects continued increasing demand for our products, an increase in royalty payments related to ELEVIDYS sales, as well as an increase in the write-offs of certain batches of our products not meeting our quality specifications. The majority of inventory costs for ELEVIDYS sold during the three months ended March 31, 2024 were previously expensed as research and development expense as they were incurred prior to the regulatory approval of ELEVIDYS in June 2023.

Operating expenses and others

For the three months ended March 31, 2024, research and development expenses decreased by $45.3 million compared with the same period of 2023. The changes in research and development expenses primarily reflect capitalization of commercial batches of ELEVIDYS manufactured after its approval in June 2023, partially offset by a ramp-up of our late-stage clinical trials. For the three months ended March 31, 2024, non-GAAP research and development expenses decreased by $42.6 million compared with the same period of 2023.

Selling, general and administrative expenses increased $16.3 million for the three months ended March 31, 2024 compared with the same period of 2023. The increase is primarily driven by professional services used for the launch of ELEVIDYS and ongoing litigation matters, as well as the timing of charitable contributions. Non-GAAP selling, general and administrative expenses increased $17.2 million for the three months ended March 31, 2024 compared with the same period of 2023.

For the three months ended March 31, 2024, other income, net decreased by $6.2 million compared with the same period of 2023, which primarily reflects an increase in the fair value of our contingent consideration liability.

Income tax expense for the three months ended March 31, 2024 and 2023, was approximately $5.3 million and $4.0 million, respectively. Income tax expense for all periods presented primarily relates to federal and state income taxes.

Use of Non-GAAP Measures

In addition to the GAAP financial measures set forth in this press release, we have included certain non-GAAP measurements. The non-GAAP income (loss) is defined by us as GAAP net income (loss) excluding interest income, net, depreciation and amortization expense, stock-based compensation expense, the estimated income tax impact of each pre-tax non-GAAP adjustment and other items. The non-GAAP operating income (loss) is defined as GAAP operating income (loss) excluding depreciation and amortization expense, stock-based compensation expense and other items. Non-GAAP research and development expenses are defined by us 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 us as GAAP selling, general and administrative expenses excluding depreciation expense, stock-based compensation expense and other items.

1. Interest, depreciation and amortization

Interest income, net 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 our operations. 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 our operating performance. Amortization expense primarily associated with patent costs are amortized over a period of several years after acquisition or patent application or renewal.

2. Stock-based compensation expenses

Stock-based compensation expenses represent non-cash charges related to equity awards we have granted. Although these are recurring charges to operations, we believe 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 our control. Therefore, we believe that excluding these charges facilitates comparisons of our operational performance in different periods.

3. Other items

We evaluate other items of expense and income on an individual basis. We take into consideration quantitative and qualitative characteristics of each item, including (a) nature, (b) whether the items relate to our ongoing business operations, and (c) whether we expect the items to continue or occur on a regular basis. These other items include impairment of strategic investments, change in fair value of contingent consideration, net, gain from sale of the PRV and loss on debt extinguishment and may include other items that fit the above characteristics in the future.

- We exclude from our non-GAAP results the impairment of any strategic investments as it is a non-cash item and is not considered to be a normal operating expense due to the variability of amount and lack of predictability as to the occurrence and/or timing of such impairments.
- We exclude from our non-GAAP results the loss on debt extinguishment, which is considered to be an infrequent and non-cash event as it is associated with a distinct financing decision and is not indicative of the performance of our core operations, which accordingly, would make it difficult to compare our results to peer companies that also provide non-GAAP disclosures.
- We exclude from our non-GAAP results the change in fair value of contingent consideration, net related to regulatory-related contingent payments meeting the definition of a derivative to Myonexus selling shareholders as well as to academic institutions under separate license agreements as it is a non-cash item and is not considered to be normal operating expenses due to its variability of amounts and lack of predictability as to occurrence and/or timing.

Beginning in the fourth quarter of 2023, amortization of in-licensed rights (formerly included within depreciation and amortization expense) and income tax (benefit) expense are no longer excluded from the non-GAAP results. We now include the income tax effect of adjustments, which represents the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable effective income tax rate. Non-GAAP financial results for the first quarter of 2023 have been updated to reflect this change for comparability.

We use these non-GAAP measures as key performance measures for the purpose of evaluating operational performance and cash requirements internally. We also believe these non-GAAP measures increase comparability of period-to-period results and are useful to investors as they provide a similar basis for evaluating our performance as is applied by management. These non-GAAP measures are not intended to be considered in isolation or to replace the presentation of our 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 net income (loss), and non-GAAP diluted net earnings (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 bind to exon 51 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 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 production in skeletal muscle observed in some patients treated with EXONDYS 51. 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, bronchospasm, chest pain, cough, tachycardia, and urticaria 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.

The most common adverse reactions from observational clinical studies (N=163) seen in greater than 10% of patients were headache, cough, rash, and vomiting.

For further information, please see the full Prescribing Information.

About VYONDYS 53

VYONDYS 53 (golodirsen) 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 (DMD) 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 for this indication 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.

Kidney toxicity was observed in animals who received golodirsen. Although kidney toxicity was not observed in the clinical studies with VYONDYS 53, the clinical experience with VYONDYS 53 is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney 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 kidney function in DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting VYONDYS 53. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting VYONDYS 53. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio every three months. Only urine expected to be free of excreted VYONDYS 53 should be used for monitoring of urine protein. Urine obtained on the day of VYONDYS 53 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any VYONDYS 53 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

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 AMONDYS 45

AMONDYS 45 (casimersen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 45 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 45 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

AMONDYS 45 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 45 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

AMONDYS 45 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information for AMONDYS 45

CONTRAINDICATION:

Known hypersensitivity to casimersen or any of the inactive ingredients. Instances of hypersensitivity including angioedema and anaphylaxis have occurred.

WARNINGS AND PRECAUTIONS

Hypersensitivity: Hypersensitivity reactions, including angioedema and anaphylaxis, have occurred in patients who were treated with AMONDYS 45. If a hypersensitivity reaction occurs, institute appropriate medical treatment, and consider slowing the infusion, interrupting, or discontinuing the AMONDYS 45 infusion and monitor until the condition resolves.

Kidney Toxicity: Kidney toxicity was observed in animals who received casimersen. Although kidney toxicity was not observed in the clinical studies with AMONDYS 45, kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides. Kidney function should be monitored in patients taking AMONDYS 45. Because of the effect of reduced skeletal muscle mass on creatinine measurements, creatinine may not be a reliable measure of kidney function in DMD patients. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting AMONDYS 45. Consider also measuring glomerular filtration rate using an exogenous filtration marker before starting AMONDYS 45. During treatment, monitor urine dipstick every month, and serum cystatin C and urine protein-to-creatinine ratio (UPCR) every three months. Only urine expected to be free of excreted AMONDYS 45 should be used for monitoring of urine protein. Urine obtained on the day of AMONDYS 45 infusion prior to the infusion, or urine obtained at least 48 hours after the most recent infusion, may be used. Alternatively, use a laboratory test that does not use the reagent pyrogallol red, as this reagent has the potential to cross react with any AMONDYS 45 that is excreted in the urine and thus lead to a false positive result for urine protein.

If a persistent increase in serum cystatin C or proteinuria is detected, refer to a pediatric nephrologist for further evaluation.

Adverse Reactions: Adverse reactions occurring in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were (AMONDYS 45, placebo): upper respiratory infections (65%, 55%), cough (33%, 26%), pyrexia (33%, 23%), headache (32%, 19%), arthralgia (21%, 10%), and oropharyngeal pain (21%, 7%).

Other adverse reactions that occurred in at least 10% of patients treated with AMONDYS 45 and at least 5% more frequently than in the placebo group were: ear pain, nausea, ear infection, post-traumatic pain, and dizziness and light-headedness.

For further information, please see the full Prescribing Information.

About ELEVIDYS (delandistrogene moxeparvovec-rokl)

ELEVIDYS (delandistrogene moxeparvovec-rokl) is a single-dose, adeno-associated virus (AAV) based gene transfer therapy for intravenous infusion designed to address the underlying genetic cause of Duchenne muscular dystrophy – mutations or changes in the dystrophin gene that result in the lack of dystrophin protein – through the delivery of a transgene that codes for the targeted production of ELEVIDYS micro-dystrophin in skeletal muscle. ELEVIDYS is a one-time infusion indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene and is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin in skeletal muscle observed in patients treated with ELEVIDYS. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. ELEVIDYS has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

ELEVIDYS has been evaluated in four clinical studies: SRP-9001-101, SRP-9001-102, SRP-9001-103 (ENDEAVOR) and SRP-9001-301 (EMBARK). Accelerated approval of ELEVIDYS was primarily based on data from SRP-9001-102 and SRP-9001-103. The EMBARK study serves as the postmarketing confirmatory trial.

Important Safety Information for ELEVIDYS

CONTRAINDICATION:

ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.

WARNINGS AND PRECAUTIONS:

Acute Serious Liver Injury:

- Acute serious liver injury has been observed with ELEVIDYS. Administration of ELEVIDYS may result in elevations of liver enzymes (e.g., GGT, GLDH, ALT, AST) or total bilirubin, typically seen within 8 weeks.
- Patients with preexisting liver impairment, chronic hepatic condition, or acute liver disease (e.g., acute hepatic viral infection) may be at higher risk of acute serious liver injury. Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled.
- Prior to ELEVIDYS administration, perform liver enzyme test and monitor liver function (clinical exam, GGT, and total bilirubin) weekly for the first 3 months following ELEVIDYS infusion. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam, GGT and total bilirubin levels return to near baseline levels).
- Systemic corticosteroid treatment is recommended for patients before and after ELEVIDYS infusion. Adjust corticosteroid regimen when indicated. If acute serious liver injury is suspected, a consultation with a specialist is recommended.

Immune-mediated Myositis:

- In clinical trials, immune-mediated myositis has been observed approximately 1 month following ELEVIDYS infusion in patients with deletion mutations involving exon 8 and/or exon 9 in the *DMD* gene. Symptoms of severe muscle weakness including dysphagia, dyspnea and hypophonia were observed.
- Limited data are available for ELEVIDYS treatment in patients with mutations in the *DMD* gene between exons 1 to 17 and

exons 59 to 71. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction.
- Advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea or hypophonia as these may be symptoms of myositis. Consider additional immunomodulatory treatment (immunosuppressants [e.g., calcineurin-inhibitor] in addition to corticosteroids) based on patient's clinical presentation and medical history if these symptoms occur.

Myocarditis:

- Acute serious myocarditis and troponin-I elevations have been observed following ELEVIDYS infusion in clinical trials.
- Monitor troponin-I before ELEVIDYS infusion and weekly for the first month following infusion and continue monitoring if clinically indicated. More frequent monitoring may be warranted in the presence of cardiac symptoms, such as chest pain or shortness of breath.
- Advise patients to contact a physician immediately if they experience cardiac symptoms.

Pre-existing Immunity against AAVrh74:

- In AAV-vector based gene therapies, preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels. Following treatment with ELEVIDYS, all subjects developed anti-AAVrh74 antibodies.
- Perform baseline testing for the presence of anti-AAVrh74 total binding antibodies prior to ELEVIDYS administration.
- ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers greater than or equal to 1:400.

Adverse Reactions:

- The most common adverse reactions (incidence ≥ 5%) reported in clinical studies were vomiting, nausea, liver function test increased, pyrexia, and thrombocytopenia.

Sarepta is responsible for global development and manufacturing for ELEVIDYS, and distribution within the U.S. will commence immediately. In December 2019, Sarepta partnered with Roche to accelerate access to ELEVIDYS for patients outside the United States.

ELEVIDYS is approved under accelerated review based on expression of ELEVIDYS micro-dystrophin in skeletal muscle. Continued approval for this indication in this and other age groups will be contingent upon verification of a clinical benefit in confirmatory trials. ELEVIDYS has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on LinkedIn, X (formerly Twitter), 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 future operations, financial performance and projections, business plans, market opportunities, priorities and research and development programs and technologies; the potential benefits of our technologies and scientific approaches; the potential to bring a better life to the patient communities we serve and for 2024 to be the most profound year yet in the fight against the effects of Duchenne muscular dystrophy and a bellwether for the transformative potential of gene therapy for rare disease; our understanding that FDA does not plan to hold an advisory committee regarding the ELEVIDYS expansion; the potential for our comprehensive approach of measuring total antibodies to help improve the safety and efficacy of AAV-based gene transfer therapies; and milestones and plans, including working with FDA to expand the ELEVIDYS addressable population, with a target action date of June 21, 2024.

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 comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; certain programs may never advance in the clinic or may be discontinued for a number of reasons, including regulators imposing a clinical hold and us suspending or terminating clinical research or trials; 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; 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, 2023 and our 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.

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 March 31,	
	2024	**2023**
Revenues:		
Products, net	$ 359,484	$ 231,495
Collaboration and other	53,980	22,005
Total revenues	413,464	253,500
Cost and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	50,559	35,017
Research and development	200,396	245,679
Selling, general and administrative	127,003	110,714
Amortization of in-licensed rights	601	178
Total cost and expenses	378,559	391,588
Operating income (loss)	34,905	(138,088)
Other income (loss), net:		
Loss on debt extinguishment	—	(387,329)
Other income, net	6,543	12,707
Total other income (loss), net	6,543	(374,622)
Income (loss) before income tax expense	41,448	(512,710)
Income tax expense	5,329	4,045
Net income (loss)	$ 36,119	$ (516,755)
Net earnings (loss) per share:		
Basic	$ 0.38	$ (5.86)
Diluted	$ 0.37	$ (5.86)
Weighted average number of shares of common stock used in computing net earnings (loss) per share:		
Basic	93,991	88,186
Diluted	99,114	88,186

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

	For the Three Months Ended March 31,	
	2024	**2023**
GAAP net income (loss)	$ 36,119	$ (516,755)
Interest income, net	(15,731)	(12,992)
Depreciation and amortization expense*	8,143	11,127
Stock-based compensation expense	40,692	41,250
Change in fair value on contingent consideration	10,100	—
Loss on debt extinguishment	—	387,329
Impairment of strategic investments	—	321
Income tax effect of adjustments**	(1,083)	1,983
Non-GAAP net income (loss)**	$ 78,240	$ (87,737)

GAAP net earnings (loss) per share - diluted:	$ 0.37	$ (5.86)
Add: impact of GAAP to Non-GAAP adjustments	0.36	4.87
Non-GAAP net earnings (loss) per share - diluted***	$ 0.73	$ (0.99)
Weighted average number of shares of common stock used in computing diluted earnings (loss) per share:****		
GAAP	99,114	88,186
Non-GAAP	107,215	88,186

*Beginning in the fourth quarter of 2023, depreciation and amortization excludes amortization of in-licensed rights. Non-GAAP financial results for the first quarter 2023 have been updated to reflect this change for comparability.

**Beginning in the fourth quarter of 2023, income tax (benefit) expense is no longer excluded from the non-GAAP results. We have replaced this metric with income tax effect of adjustments, which represents the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable statutory income tax rate. Refer below for a reconciliation of effective tax rates. Non-GAAP financial results for the first quarter 2023 have been updated to reflect this change for comparability.

***Non-GAAP earnings per share is calculated using diluted shares whereas non-GAAP net loss per share is calculated using basic shares as all other instruments are anti-dilutive.

****The difference between the weighted average number of shares of common stock used in computing diluted GAAP and non-GAAP earnings per share for the three months ended March 31, 2024, is a result of the exclusion of the potential share settlement of the 2027 Notes from the GAAP earnings per share as the inclusion of such shares was anti-dilutive.

	For the Three Months Ended March 31,	
	2024	**2023**
Total effective tax rate, GAAP	14.2%	(0.8)%
Less: impact of GAAP to Non-GAAP adjustments	(5.4)	(1.6)
Total effective tax rate, Non-GAAP	8.8%	(2.4)%

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

	For the Three Months Ended March 31,	
	2024	**2023**
GAAP research and development expenses	$ 200,396	$ 245,679
Stock-based compensation expense	(16,273)	(16,413)
Depreciation and amortization expense	(6,046)	(8,551)
Non-GAAP research and development expenses	$ 178,077	$ 220,715

	For the Three Months Ended March 31,	
	2024	**2023**
GAAP selling, general and administrative expenses	$ 127,003	$ 110,714
Stock-based compensation expense	(24,419)	(24,837)
Depreciation expense	(2,097)	(2,576)
Non-GAAP selling, general and administrative expenses	$ 100,487	$ 83,301

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

	As of March 31, 2024	As of December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 427,290	$ 428,430
Short-term investments	963,453	1,247,820
Accounts receivable, net	378,806	400,327
Inventory	373,530	322,859
Manufacturing-related deposits and prepaids	238,821	102,181
Other current assets	82,965	77,714

Total current assets	2,464,865	2,579,331
Property and equipment, net	249,302	227,154
Right of use assets	126,269	129,952
Non-current inventory	207,542	191,368
Other non-current assets	176,407	136,771
Total assets	$ 3,224,385	$ 3,264,576
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 91,536	$ 164,918
Accrued expenses	283,317	314,997
Deferred revenue, current portion	112,000	50,416
Current portion of long-term debt	105,586	105,483
Other current liabilities	16,270	17,845
Total current liabilities	608,709	653,659
Long-term debt	1,133,660	1,132,515
Lease liabilities, net of current portion	140,102	140,965
Deferred revenue, net of current portion	325,000	437,000
Contingent consideration	48,200	38,100
Other non-current liabilities	7,522	3,000
Total liabilities	2,263,193	2,405,239
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 198,000,000 shares authorized; 94,490,157 and 93,731,831 issued and outstanding at March 31, 2024 and December 31, 2023, respectively	9	9
Additional paid-in capital	5,371,968	5,304,623
Accumulated other comprehensive (loss) income, net of tax	(691)	918
Accumulated deficit	(4,410,094)	(4,446,213)
Total stockholders' equity	961,192	859,337
Total liabilities and stockholders' equity	$ 3,224,385	$ 3,264,576

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