Rhythm Pharmaceuticals Reports Third Quarter 2022 Financial Results and Business Update

November 8, 2022

-- U.S. launch of IMCIVREE® (setmelanotide) for Bardet-Biedl Syndrome progresses with strong demand with more than 120 prescriptions since FDA approval --

-- EC authorization for IMCIVREE expanded to include BBS; UK launch in POMC and LEPR deficiencies underway --

-- Setmelanotide received FDA Breakthrough Therapy Designation for hypothalamic obesity; Phase 3 trial expected to be initiated in early 2023 --

-- Successfully completed $140 million public offering with exercise of underwriters' option, extending cash runway into 2025 --

-- Management to host conference call today at 8:00 a.m. ET --

BOSTON, Nov. 08, 2022 (GLOBE NEWSWIRE) -- Rhythm Pharmaceuticals, Inc. (Nasdaq: RYTM) today reported financial results and provided a business update for the third quarter ended September 30, 2022.

"The third quarter of 2022 was transformative for Rhythm and our global strategy to deliver IMCIVREE ® (setmelanotide) as the first precision medicine to address the needs of patients and families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) pathway diseases," said David Meeker, M.D., Chair, President and Chief Executive Officer of Rhythm. "We are pleased with our U.S. commercial launch and the initial reception of IMCIVREE for Bardet-Biedl syndrome (BBS), and we look forward to continuing to build on early market access successes in Europe."

Dr. Meeker continued, "We also are excited by the potential of setmelanotide to achieve a profound, consistent and sustained reduction in body weight in patients with hypothalamic obesity as demonstrated in our Phase 2 and long-term-extension trials. Following recent feedback from the U.S. Food and Drug Administration (FDA), we look forward to initiating our pivotal Phase 3 trial early in 2023."

Third Quarter and Recent Business Highlights:

Development, Regulatory and Commercial Updates:
Hypothalamic Obesity

- In November 2022, Rhythm announced that it had reached alignment with the FDA on the design of its Phase 3 clinical trial to evaluate setmelanotide for the treatment of acquired hypothalamic obesity. The trial, which is anticipated to initiate in early 2023, is expected to enroll 120 patients randomized 2:1 to setmelanotide therapy or placebo. The primary endpoint will be the percent change in body mass index (BMI) from baseline to after approximately 52 weeks on a therapeutic regimen of setmelanotide versus placebo.
- Also in November 2022, the Company announced that the FDA has granted Breakthrough Therapy Designation to setmelanotide for the treatment of hypothalamic obesity.
- In November 2022, at The Obesity Society's ObesityWeek ®, Rhythm and its collaborators delivered a total of 11 presentations, including the full dataset from the Phase 2 clinical trial evaluating setmelanotide in hypothalamic obesity, as well as data from patients with hypothalamic obesity who enrolled into the long-term extension trial. Sixteen of 18 patients (89%) achieved the primary endpoint with a 5% or greater reduction in BMI and 14 of 18 patients (78%) achieved a 10% or greater reduction in BMI. Fourteen patients from this Phase 2 trial enrolled in Rhythm's long-term extension trial, and, as of a cut-off date of September 23, 2022, 13 patients achieved 21.1% mean reduction in BMI at 29 weeks and 5 of these patients achieved 26.7% mean reduction at 41 weeks.

Bardet-Biedl Syndrome

- Today, Rhythm announced that, as of September 30, 2022, more than 80 physicians have written in total more than 120 prescriptions for IMCIVREE for patients with BBS since IMCIVREE was approved by the FDA; Rhythm has secured approval for reimbursement for more than 40 of those prescriptions.
- In September 2022, Rhythm announced that the European Commission (EC) expanded marketing authorization for IMCIVREE to include the treatment of obesity and control of hunger associated with genetically confirmed BBS in adult and pediatric patients 6 years of age and older.

- Also in September, Rhythm announced that Health Canada granted Priority Review for the Company's New Drug Submission (NDS) for setmelanotide, indicated in adult and pediatric patients 6 years of age and older with impairments in the MC4R pathway due to genetic diseases, for the treatment of obesity and control of hunger in BBS or biallelic pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency.

POMC and LEPR Deficiency Obesities

- In October 2022, Rhythm launched IMCIVREE in the United Kingdom for patients with POMC, PCSK1 or LEPR deficiency obesity.

Clinical Development Updates:

- In October 2022, Rhythm announced its sponsorship of the inaugural International Meeting on Pathway-Related Obesity: Vision of Excellence (IMPROVE) 2022, a scientific meeting for health care professionals based in Europe to discuss and share the latest scientific developments and patient care practices related to rare pathway-related obesities, including those driven by impairments in the MC4R pathway, in which genetic deficiencies can result in hyperphagia and early onset severe obesity. At IMPROVE 2022, Rhythm presented new data from its exploratory Phase 2 Basket Study evaluating setmelanotide in patients with predicted rescuable or nonrescuable MC4R obesity based on an *in vitro* assay, which suggested that the assay used to classify MC4R variants had limited predictive value in identifying the approximately 20% of patients who met the definition for setmelanotide response. The Company intends to continue assessment of the genetics of the MC4R pathway and opportunities to identify patients who may benefit from setmelanotide.
- In September 2022 at the 60th Annual Meeting of the European Society for Paediatric Endocrinology (ESPE 2022), Rhythm and its collaborators presented new findings on the burden of hyperphagia and obesity on patients with BBS and their caregivers and data from new analyses that showed setmelanotide achieved substantial weight loss benefit in adolescent and pediatric patients with rare MC4R pathway diseases across three separate pivotal trials.

Corporate:

- In October 2022, Rhythm announced the appointment of Dana Washburn, M.D., as Senior Vice President of Clinical Development and a member of the Company's Executive Leadership Team.
- In September 2022, Rhythm announced the publication of a children's book titled, "Understanding Hunger & Bardet-Biedl Syndrome (BBS): Gabe's Story," developed in collaboration with the Bardet-Biedl Syndrome (BBS) Foundation. "Gabe's Story" is designed to help children living with BBS make sense of their feelings, provide education on why they are experiencing insatiable hunger, and recognize they are not alone.
- In September 2022, Rhythm completed a public offering of 4,800,000 shares of its common stock at a price to the public of $26.00 per share for aggregate net proceeds of $117.0 million, after deducting underwriting discounts and commissions and offering expenses. On October 18, 2022, the Company completed the sale of an additional 580,000 shares of common stock at a price to the public of $26.00 per share pursuant to the partial exercise of the underwriters' option to purchase additional shares. The total aggregate net proceeds to Rhythm were $131.2 million after deducting underwriting discounts, commissions and offering expenses.
- In September 2022, Rhythm announced receipt of an additional investment of $37.5 million from HealthCare Royalty Partners. Under the terms of the Revenue Interest Financing Agreement with HealthCare Royalty Partners, which was announced in June 2022, Rhythm was eligible to receive this funding following EC marketing authorization for IMCIVREE for BBS. The Company remains eligible for an additional investment amount of $25.0 million, which would be payable upon the achievement of certain agreed sales milestones in 2023.

Key Upcoming Milestones:
Rhythm expects to achieve the following near-term milestones:

- Launch IMCIVREE in Italy and the Netherlands for patients with POMC, PCSK1 or LEPR deficiencies in the fourth quarter of 2022.
- Initiate a pivotal Phase 3 trial to evaluate setmelanotide in hypothalamic obesity in early 2023.
- Initiate a Phase 3, randomized, double-blind trial in patients naïve to setmelanotide therapy ("de novo study") to evaluate the weekly formulation of setmelanotide in patients with BBS in the first half of 2023.
- Complete regulatory review by Health Canada and, pending approval, make IMCIVREE commercially available in Canada for the treatment of obesity and control of hunger in adults and pediatric patients 6 years and older with BBS, or with POMC, PCSK1 or LEPR deficiencies in 2023.
- Announce topline data from the ongoing Phase 3, open-label trial evaluating one year of setmelanotide therapy in pediatric patients with MC4R pathway deficiencies between the ages of 2 and 6 years old in the second half of 2023.

Third Quarter 2022 Financial Results:

- **Cash Position:** As of September 30, 2022, cash, cash equivalents and short-term investments were approximately $347.8 million, as compared to $294.9 million as of December 31, 2021. This increase includes net proceeds of $117.0 million received upon the closing of Rhythm's public offering in September 2022 and an additional investment of $37.5 million from HealthCare Royalty, which Rhythm received following the European Commission's expansion of marketing authorization for IMCIVREE to include patients with BBS, partially offset by cash used to fund operating activities in 2022. The October 2022 partial exercise of the underwriters' option to purchase additional shares resulted in additional net

proceeds of $14.2 million, which is not reflected in Rhythm's September 30, 2022 cash and cash equivalents and short-term investments balance.

- **Revenue:** Product revenues, net, relating to sales of IMCIVREE were $4.3 million for the third quarter of 2022, as compared to $1.0 million for the third quarter of 2021.
- **Cost of sales:** Cost of sales was $0.5 million for the third quarter of 2022, as compared to $0.2 million for third quarter of 2021. This increase primarily reflects the amortization of a capitalized sales-based milestone payment made to Ipsen Pharma S.A.S., or Ipsen, upon first commercial sale in the United States and European Union, as well as a royalty due to Ipsen on net product sales.
- **R&D Expenses:** R&D expenses were $21.1 million in the third quarter of 2022, as compared to $27.5 million in the third quarter of 2021. The year-over-year decrease was due to a decrease of $5.2 million in clinical trial costs associated with the impact of study design amendments to the Phase 2 DAYBREAK study and reduced activity due to the winding down of Rhythm's BBS, QTc, Phase 2 Basket and renal studies; these decreases were partially offset by increased costs associated with the Phase 3 EMANATE trial, and increased enrollment in the long-term extension study; a decrease of $1.3 million in costs associated with the manufacturing of clinical material; a decrease of $0.5 million of costs related to next generation research and development activities; a decrease of $0.4 million in costs associated with medical affairs; and a decrease of $0.3 million in compensation and benefits. These decreases were partially offset by an increase of $1.2 million due to increased gene sequencing volumes to support expanded clinical programs.
- **S,G&A Expenses:** S,G&A expenses were $21.9 million for the third quarter of 2022, as compared to $17.5 million for the third quarter of 2021. The year-over-year increase was primarily related to an increase of $2.2 million due to increased compensation and benefits related costs associated with additional headcount to support expanding business operations as well as to build out of commercial operations in the United States and internationally; an increase of $1.6 million due to increased costs associated with insurance premiums, office support, travel and entertainment related costs for an expanding workforce and increased commercial operations; and an increase of $0.7 million related to increased costs associated with sales and marketing activities for IMCIVREE in connection with preparing for the EC approval for BBS obtained in September 2022 and expanding international market access. The above increases were partially offset by a decrease of $0.4 million related to professional services costs.
- **Other (expense) income, net:** Other (expense) income, net increased by ($1.7) million to ($1.6) million for the three months ended September 30, 2022. Other (expense) income, net consists of ($2.1) million of interest expense related to the royalty interest financing agreement (RIFA) with HealthCare Royalty Partners, ($1.0) million write off of RareStone equity, which was partially offset by $1.0 million of interest income primarily due to improving interest rates during the period and $0.7 million fair market value adjustment related to the RIFA embedded derivative.
- **Provision for income taxes.** There is no provision for income taxes for the three months ended September 30, 2022, compared to an income tax benefit of $9.0 million as a result of the reversal of a tax provision recorded upon the sale of Rhythm's priority review voucher (PRV) during the three months ended September 30, 2021.
- **Net Loss:** Net loss was $40.9 million for the third quarter of 2022, or a net loss per basic and diluted share of $0.79, as compared to a net loss of $35.1 million for the third quarter of 2021, or a net loss per basic and diluted share of $0.70.

Year to Date 2022 Financial Results:

- **Revenue:** Product revenues, net, relating to sales of IMCIVREE were $8.1 million for the nine months ended September 31, 2022, as compared to $1.3 million for the nine months ended September 30, 2021.
- **License Revenue:** License revenue relating entirely to the Company's out-license arrangement with RareStone was $6.8 million for the nine months ended September 30, 2022. There were no comparable transactions in the prior year.
- **Cost of sales:** Cost of sales was $1.1 million for the nine months ended September 30, 2022, compared to $0.4 million for the nine months ended September 30, 2021. This increase was due to $0.3 million of additional amortization of sales-based milestone payment made to Ipsen, upon first commercial sale in the United States and European Union, $0.3 million of additional royalties due to Rhythm's growth in sales, and $0.1 attributed to product cost primarily associated with higher sales volume.
- **R&D Expenses:** R&D expenses were $85.1 million for the nine months ended September 30, 2022, as compared to $72.6 million for the nine months ended September 30, 2021. The year-over-year increase was due to an increase of $8.8 million in clinical trial costs associated with new and planned clinical trials, including the Phase 2 DAYBREAK and Phase 3 EMANATE trials, Phase 3 pediatrics trial, QTc study, Phase 2 hypothalamic obesity study, and increased enrollment in the long-term extension study. These increases were partially offset by reduced activity due to the completion and winding down of the POMC and LEPR, BBS, Phase 2 Basket, renal and GO-ID studies; an increase of $3.0 million due to increased purchases of clinical supply material; an increase of $2.4 million due to increased gene sequencing costs associated with expanded clinical programs; an increase of $1.1 million in compensation and benefits due to the hiring of additional full-time employees to support the growth of research and development programs and expansion of regulatory affairs operations; an increase of $1.0 million in development milestones earned by Camurus AB, or Camurus, related to development milestone achieved related to weekly formulation; and an increase of $0.3 million related to intellectual property and patent related filing activities. These increases were partially offset by a decrease of $3.7 million in costs associated with medical affairs; and a decrease of $0.7 million in costs associated with next generation research and development activities.
- **S,G&A Expenses:** S,G&A expenses were $65.7 million for the nine months ended September 30, 2022, as compared to $47.5 million the nine months ended September 30, 2021. The year-over-year increase was primarily due to an increase of $8.1 million related to increased costs associated with commercial operations, sales and marketing activities for IMCIVREE in connection with preparing for the U.S approval for BBS obtained in June 2022 and EC approval in September 2022; an increase of $6.6 million due to increased compensation and benefits related costs associated with additional headcount to

support expanding business operations as well as to build out commercial operations in the United States and internationally; and an increase of $3.6 million due to increased costs associated with information technology, international office space, sponsorships and general corporate travel for an expanding workforce.

- **Other (expense) income, net:** Other income decreased by $101.9 million to ($1.6) million in the nine months ended September 30, 2022 as compared to the nine months ended September 30, 2022, primarily due to the sale of the PRV in February 2021. The sale of the PRV in the prior year was a non-recurring transaction. Other (expense), net consists of $2.2 million of interest expense related to the RIFA and a $1.0 million write-off of RareStone equity, which was partially offset by $1.4 million of interest income and $0.7 million fair market value adjustment related to the RIFA embedded derivative.
- **Provision for income taxes:** There was no provision for income taxes for the nine months ended September 30, 2022, as Rhythm projects to generate operating losses during the year. The Company recorded a provision for income taxes of $8.0 million as a result of the sale of its PRV during the nine months ended September 30, 2021.
- **Net Loss:** Net loss was $138.6 million for the nine months ended September 30, 2022, or a net loss per basic and diluted share of $2.73, as compared to a net loss of $26.7 million for the nine months ended September 30, 2021, or a net loss per basic and diluted share of $0.54.

Financial Guidance: Based on its current operating plans, Rhythm expects that its existing cash and cash equivalents and short-term investments as of September 30, 2022 will be sufficient to fund operations into 2025.

Conference Call Information
Rhythm Pharmaceuticals will host a live conference call and webcast at 8:00 a.m. ET today to review its third quarter 2022 financial results and recent business activities. Participants may register for the conference call here. While not required, it is recommended that participants join the call ten minutes prior to the scheduled start.

A live webcast of the call will also be available under "Events and Presentations" in the Investor Relations section of the Rhythm Pharmaceuticals website at http://ir.rhythmtx.com/. The archived webcast will be available on Rhythm Pharmaceuticals' website approximately two hours after the conference call and will be available for 30 days following the call.

About Rhythm Pharmaceuticals
Rhythm is a commercial-stage biopharmaceutical company committed to transforming the lives of patients and their families living with hyperphagia and severe obesity caused by rare melanocortin-4 receptor (MC4R) pathway diseases. Rhythm's precision medicine, setmelanotide, is approved by the U.S. Food and Drug Administration (FDA) for chronic weight management in adult and pediatric patients 6 years of age and older with monogenic or syndromic obesity due to pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency confirmed by genetic testing, or patients with a clinical diagnosis of Bardet-Biedl syndrome (BBS). The European Commission (EC) has authorized setmelanotide for the treatment of obesity and the control of hunger associated with genetically confirmed BBS or genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 6 years of age and above.

The UK's Medicines & Healthcare Products Regulatory Agency (MHRA) authorized setmelanotide for the treatment of obesity and the control of hunger associated with genetically confirmed loss-of-function biallelic POMC, including PCSK1, deficiency or biallelic LEPR deficiency in adults and children 6 years of age and above. Additionally, Rhythm is advancing a broad clinical development program for setmelanotide in other rare MC4R pathway diseases, including acquired hypothalamic obesity, and is leveraging the Rhythm Engine and the largest known obesity DNA database -- now with approximately 45,000 sequencing samples -- to improve the understanding, diagnosis and care of people living with severe obesity due to certain genetic deficiencies. Rhythm's headquarters is in Boston, MA.

Setmelanotide Indication
In the United States, setmelanotide is indicated for chronic weight management in adult and pediatric patients 6 years of age and older with monogenic or syndromic obesity due to POMC, PCSK1 or LEPR deficiency as determined by an FDA-approved test demonstrating variants in POMC, PCSK1 or LEPR genes that are interpreted as pathogenic, likely pathogenic, or of uncertain significance (VUS) or BBS.

In the European Union, setmelanotide is indicated for the treatment of obesity and the control of hunger associated with genetically confirmed Bardet-Biedl syndrome (BBS) or genetically confirmed loss-of-function biallelic pro-opiomelanocortin (POMC), including PCSK1, deficiency or biallelic leptin receptor (LEPR) deficiency in adults and children 6 years of age and above.

Limitations of Use
In the United States and Europe, Setmelanotide should be prescribed and supervised by a physician with expertise in obesity with underlying genetic etiology.

Setmelanotide is not indicated for the treatment of patients with the following conditions as setmelanotide would not be expected to be effective:

- Obesity due to suspected POMC, PCSK1 or LEPR deficiency with *POMC*, *PCSK1* or *LEPR* variants classified as benign or likely benign
- Other types of obesity not related to POMC, PCSK1 or LEPR deficiency, or BBS, including obesity associated with other genetic syndromes and general (polygenic) obesity.

WARNINGS AND PRECAUTIONS

Skin Monitoring: Setmelanotide may lead to generalized increased skin pigmentation and darkening of pre-existing naevi because of its pharmacologic effect. Full body skin examinations should be conducted annually to monitor pre-existing and new skin pigmentary lesions before and during treatment with setmelanotide.

Heart rate and blood pressure monitoring: Heart rate and blood pressure should be monitored as part of standard clinical practice at each medical visit (at least every 6 months) for patients treated with setmelanotide.

Prolonged penile erection: Spontaneous penile erections have been reported in clinical trials with setmelanotide. Patients who have a penile erection lasting longer than 4 hours should be instructed to seek emergency medical attention for potential treatment of priapism.

Depression: In clinical trials, depression has been reported in patients treated with setmelanotide. Patients with depression should be monitored at each medical visit during treatment with setmelanotide. Consideration should be given to discontinuing setmelanotide if patients experience suicidal thoughts or behaviors.

Pediatric Population: The prescribing physician should periodically assess response to setmelanotide therapy. In growing children, the impact of weight loss on growth and maturation should be evaluated. The prescribing physician should monitor growth (height and weight) using age- and sex-appropriate growth curves.

Excipients: This medicinal product contains 10 mg benzyl alcohol in each ml. Benzyl alcohol may cause allergic reactions. Patients who are pregnant or breastfeeding should be advised of the potential risk from the excipient benzyl alcohol, which might accumulate over time and cause metabolic acidosis. This medicinal product should be used with caution in patients with hepatic or renal impairment, because of the potential risk from the excipient benzyl alcohol which might accumulate over time and cause metabolic acidosis.

Sodium: This medicinal product contains less than 1 mmol sodium (23 mg) per dose, that is to say essentially “sodium-free.”

ADVERSE REACTIONS
The most frequent adverse reactions are hyperpigmentation (51%), injection site reaction (39%), nausea (33%), and headache (26%).

USE IN SPECIFIC POPULATIONS

Pregnancy
There are no data from the use of setmelanotide in pregnant women. Animal studies do not indicate direct harmful effects with respect to reproductive toxicity. However, administration of setmelanotide to pregnant rabbits resulted in decreased maternal food consumption leading to embryo-foetal effects. As a precautionary measure, setmelanotide should not be started during pregnancy or while attempting to get pregnant as weight loss during pregnancy may result in fetal harm. If a patient who is taking setmelanotide has reached a stable weight and becomes pregnant, consideration should be given to maintaining setmelanotide treatment as there was no proof of teratogenicity in the nonclinical data. If a patient who is taking setmelanotide and still losing weight gets pregnant, setmelanotide should either be discontinued, or the dose reduced while monitoring for the recommended weight gain during pregnancy. The treating physician should carefully monitor weight during pregnancy in a patient taking setmelanotide.

Breast-feeding
It is unknown whether setmelanotide is excreted in human milk. A nonclinical study showed that setmelanotide is excreted in the milk of nursing rats. No quantifiable setmelanotide concentrations were detected in plasma from nursing pups. A risk to the newborn/infant cannot be excluded. A decision must be made whether to discontinue breastfeeding or to discontinue/abstain from setmelanotide therapy taking into account the benefit of breastfeeding for the child and the benefit of therapy for the mother.

Fertility
No human data on the effect of setmelanotide on fertility are available. Animal studies did not indicate harmful effects with respect to fertility.

To report SUSPECTED ADVERSE REACTIONS, contact Rhythm Pharmaceuticals at +1 (833) 789-6337. See Summary of Product Characteristics’ APPENDIX V for a list of European national reporting systems to communicate adverse reactions.

Please see the full Prescribing Information for additional Important Safety Information.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding the potential, safety, efficacy, and regulatory and clinical progress of setmelanotide, including the anticipated timing for initiation of clinical trials and release of clinical trial data, our expectations surrounding potential regulatory submissions, approvals and timing thereof, our business strategy and plans, including regarding commercialization of setmelanotide in Europe, the United Kingdom, Canada, the United States and other international regions, our ability to reach certain milestones and receive additional investment amounts under our financing agreement with HealthCare

Royalty, our participation in upcoming events and presentations, the intended use of proceeds from our public offering, management changes, and the sufficiency of our cash, cash equivalents and short-term investments to fund our operations. Statements using word such as "expect", "anticipate", "believe", "may", "will" and similar terms are also forward-looking statements. Such statements are subject to numerous risks and uncertainties, including, but not limited to, the impact of our management transition, our ability to enroll patients in clinical trials, the design and outcome of clinical trials, the impact of competition, the ability to achieve or obtain necessary regulatory approvals, risks associated with data analysis and reporting, our liquidity and expenses, the impact of the COVID-19 pandemic on our business and operations, including our preclinical studies, clinical trials and commercialization prospects, and general economic conditions, and the other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022 and our other filings with the Securities and Exchange Commission. Except as required by law, we undertake no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

Rhythm Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations and Comprehensive (Loss) Income
(in thousands, except share and per share data)
(Unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2022	2021	2022	2021
Product revenue, net	$ 4,284	$ 1,028	$ 8,094	$ 1,337
License revenue	—	—	6,754	—
Costs and expenses:				
Cost of sales	497	222	1,105	363
Research and development	21,116	27,539	85,082	72,554
Selling, general, and administrative	21,938	17,507	65,715	47,490
Total costs and expenses	43,551	45,268	151,902	120,407
Loss from operations	(39,267)	(44,240)	(137,054)	(119,070)
Other income:				
Other expense	(370)	—	(370)	100,000
Interest expense	(2,144)	—	(2,190)	—
Interest income	920	138	988	313
Total other (expense) income, net	(1,594)	138	(1,572)	100,313
Loss before taxes	(40,861)	(44,102)	(138,626)	(18,757)
(Benefit from) provision for income taxes	—	(8,995)	—	7,989
Net loss	$ (40,861)	$ (35,107)	$ (138,626)	$ (26,746)
Net loss per share, basic and diluted	$ (0.79)	$ (0.70)	$ (2.73)	$ (0.54)
Weighted-average common shares outstanding, basic and diluted	51,400,922	50,246,303	50,712,452	49,374,336
Other comprehensive (loss) income:				
Net loss	$ (40,861)	$ (35,107)	$ (138,626)	$ (26,746)
Unrealized loss, net on marketable securities and other long-term assets	567	—	(338)	(107)
Comprehensive loss	$ (40,294)	$ (35,107)	$ (138,964)	$ (26,853)

Rhythm Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	September 30, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 185,087	$ 59,248
Short-term investments	162,708	235,607
Accounts receivable, net	3,328	1,025
Prepaid expenses and other current assets	10,751	12,507

Total current assets	361,874	308,387
Property and equipment, net	2,426	2,813
Right-of-use asset	1,273	1,522
Intangible assets, net	8,097	4,658
Restricted cash	328	328
Other long-term assets	15,616	11,815
Total assets	$ 389,614	$ 329,523
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,933	$ 5,737
Accrued expenses and other current liabilities	25,741	30,084
Deferred revenue	1,760	7,000
Lease liability	664	606
Total current liabilities	33,098	43,427
Long-term liabilities:		
Deferred royalty obligation	72,961	—
Lease liability	1,440	1,945
Derivative liability	920	—
Total liabilities	108,419	45,372
Stockholders' equity:		
Preferred Stock, $0.001 par value: 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2022 and December 31, 2021	—	—
Common stock, $0.001 par value: 120,000,000 shares authorized; 55,756,256 and 50,283,574 shares issued and outstanding at September 30, 2022 and December 31, 2021, respectively	56	50
Additional paid-in capital	949,043	813,041
Accumulated other comprehensive loss	(339)	(1)
Accumulated deficit	(667,565)	(528,939)
Total stockholders' equity	281,195	284,151
Total liabilities and stockholders' equity	$ 389,614	$ 329,523

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