Travere Therapeutics Reports Third Quarter 2021 Financial Results and Organizational Progress

October 28, 2021

Recently reported positive topline interim results from the ongoing pivotal Phase 3 PROTECT Study of sparsentan in IgA nephropathy

Following pre-NDA interactions with FDA, NDA submission of sparsentan in IgA nephropathy for accelerated approval under Subpart H expected in the first quarter of 2022

Based on FDA interactions, accelerated approval submission of sparsentan in FSGS planned for mid-2022, pending additional supportive eGFR data from the ongoing DUPLEX Study

Joint collaboration with Vifor Pharma to commercialize sparsentan in Europe; combined IgA nephropathy and FSGS MAA submission for sparsentan in Europe expected mid-2022

Net product sales of $54.2 million for the third quarter of 2021

SAN DIEGO, Oct. 28, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today reported its third quarter 2021 financial results and provided a corporate update.

- In August 2021, the Company reported that the ongoing pivotal Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN) met its pre-specified interim primary efficacy endpoint, demonstrating a greater than threefold reduction of proteinuria from baseline after 36 weeks of treatment, compared to the active control irbesartan ($p<0.0001$)
- In its recent pre-New Drug Application (NDA) interactions with the U.S. Food and Drug Administration (FDA), the Company confirmed that the interim results from the ongoing PROTECT Study of sparsentan in IgAN support an application for accelerated approval under Subpart H in the U.S.; NDA submission is expected in the first quarter of 2022
- In September 2021, the Company announced alignment with FDA on a pathway to proceed with a Subpart H NDA submission for accelerated approval of sparsentan in focal segmental glomerulosclerosis (FSGS) in mid-2022, pending additional supportive estimated glomerular filtration rate (eGFR) data from the ongoing DUPLEX Study
- The Company has formalized plans for a combined IgAN and FSGS Marketing Authorization Application (MAA) for sparsentan in Europe; submission is expected in mid-2022
- In September 2021, the Company entered into a joint collaboration and licensing agreement with Vifor Pharma for the commercialization of sparsentan in Europe, Australia and New Zealand
- Ruth Williams-Brinkley joined the Board of Directors, bringing to Travere more than 35 years of executive leadership in care delivery and health plan operations
- Net product sales for the third quarter 2021 were $54.2 million, compared to $51.1 million for the same period in 2020
- Cash, cash equivalents and marketable securities, as of September 30, 2021, totaled $551.2 million, which includes the $55.0 million upfront payment from the joint collaboration and licensing agreement with Vifor Pharma

“The third quarter was pivotal for our sparsentan development programs. In August, we announced impressive topline interim results from the ongoing Phase 3 PROTECT Study of sparsentan in IgA nephropathy, and we recently concluded successful pre-NDA interactions with the FDA that put us on track for an NDA submission for accelerated approval in the first quarter of next year,” said Eric Dube, Ph.D., chief executive officer of Travere Therapeutics. “We also made regulatory progress that positions us for additional submissions mid-next year, including a potential NDA submission for accelerated approval of sparsentan for FSGS, and a combined IgA nephropathy and FSGS MAA submission for conditional marketing authorization in Europe. We look forward to continuing our strong execution through the balance of 2021, and to building upon the strength of our existing commercial organization to prepare for multiple potential launches of sparsentan, if approved.”

Quarter Ended September 30, 2021

Net product sales for the third quarter of 2021 were $54.2 million, compared to $51.1 million for the same period in 2020. For the nine months ended September 30, 2021, net product sales were $156.2 million, compared to $147.3 million for the same period in 2020. The increase in net product sales was attributable to growth across the Company’s commercial products.

Research and development (R&D) expenses for the third quarter of 2021 were $48.4 million, compared to $32.3 million for the same period in 2020. For the nine months ended September 30, 2021, R&D expenses were $148.2 million, compared to $93.4 million for the same period in 2020. The difference is largely attributable to the fully enrolled and ongoing pivotal DUPLEX and PROTECT studies of sparsentan, as well as the continued development of the pegtibatinase program in classical homocystinuria (HCU). On a non-GAAP adjusted basis, R&D expenses were $45.2 million for the third quarter of 2021, compared to $29.5 million for the same period in 2020.

Selling, general and administrative (SG&A) expenses for the third quarter of 2021 were $36.1 million, compared to $32.0 million for the same period in 2020. For the nine months ended September 30, 2021, SG&A expenses were $107.8 million, compared to $100.1 million for the same period in 2020. The difference is largely attributable to increased headcount as a result of the Company’s operational growth, and professional fees. On a non-GAAP adjusted basis, SG&A expenses were $25.5 million for the third quarter of 2021, compared to $22.9 million for the same period in 2020.

Total other expense, net, for the third quarter of 2021 was $3.9 million, compared to $3.1 million for the same period in 2020. The difference is largely attributable to a reduction in interest income.

Net loss for the third quarter of 2021 was $35.6 million, or $0.59 per basic share, compared to a net loss of $22.5 million, or $0.44 per basic share for the same period in 2020. For the nine months ended September 30, 2021, net loss was $128.5 million, compared to $47.8 million for the same period in 2020. On a non-GAAP adjusted basis, net loss for the third quarter of 2021 was $7.9 million, or $0.13 per basic share, compared to a net loss of $5.6 million, or $0.11 per basic share for the same period in 2020.

As of September 30, 2021, the Company had cash, cash equivalents and marketable securities of $551.2 million. This includes the $55.0 million upfront payment received from the joint collaboration and licensing agreement entered into with Vifor Pharma during the third quarter.

Program Updates

Sparsentan - IgAN

- In August 2021, the Company announced positive topline interim results from the ongoing pivotal Phase 3 PROTECT Study of sparsentan in IgAN. The PROTECT Study met its pre-specified interim primary efficacy endpoint with statistical significance. After 36 weeks of treatment, patients receiving sparsentan achieved a mean reduction in proteinuria from baseline of 49.8 percent, compared to a mean reduction in proteinuria from baseline of 15.1 percent for irbesartan-treated patients ($p<0.0001$). The Company believes that preliminary eGFR data available at the time of the interim analysis are indicative of a potential clinically meaningful treatment effect after two years of treatment. Preliminary results at the time of the interim assessment suggested that sparsentan had been generally well-tolerated to date in the study and consistent with its overall observed safety profile. The PROTECT Study is fully enrolled and is scheduled to continue as planned on a blinded basis to assess the treatment effect on eGFR slope over 110 weeks in the confirmatory endpoint analysis. Topline results from the confirmatory endpoint analysis are expected in the second half of 2023.

- The Company recently completed successful pre-NDA interactions with the FDA for sparsentan in IgAN. The FDA concurred that the interim analyses from the PROTECT Study support submission of an application for accelerated approval under Subpart H. The Company expects to submit an NDA for accelerated approval of sparsentan for IgAN in the first quarter of 2022.

- In mid-2022, the Company expects to submit a combined IgAN and FSGS MAA application for conditional marketing authorization of sparsentan in Europe.

Sparsentan - FSGS

- In February 2021, the Company announced that the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in FSGS achieved its pre-specified interim FSGS partial remission of proteinuria endpoint (FPRE) with statistical significance. FPRE is a clinically meaningful endpoint defined as urine protein-to-creatinine ratio (UP/C) ≤1.5 g/g and a >40 percent reduction in UP/C from baseline. After 36 weeks of treatment, 42.0 percent of patients receiving sparsentan achieved FPRE, compared to 26.0 percent of irbesartan-treated patients ($p=0.0094$). Preliminary results at the time of the interim assessment suggested that sparsentan had been generally well-tolerated and shown a comparable safety profile to irbesartan. The DUPLEX Study is fully enrolled and scheduled to continue as planned on a blinded basis to assess the confirmatory eGFR endpoint after 108 weeks of treatment. Topline results from the confirmatory endpoint are expected in the first half of 2023.

- During the third quarter of 2021, the Company announced a successful outcome from its Type A meeting with the FDA in which alignment was reached on the Company's plan to provide additional eGFR data from the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in FSGS to support a potential NDA submission for accelerated approval. The Company plans to submit an NDA for accelerated approval in the U.S. in mid-2022, should additional eGFR data from the study be supportive, as expected.

Pegtibatinase (TVT-058)

- The ongoing Phase 1/2 COMPOSE Study, a dose escalating clinical trial to assess the safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects of pegtibatinase in patients with classical HCU, continues to progress. The Company anticipates preliminary data from the COMPOSE Study to become available in 2021 and is monitoring the potential impact of the evolving COVID-19 pandemic on this timing. If ultimately approved, pegtibatinase has the potential to become the first disease modifying therapy for people living with classical HCU.

Joint Collaboration and Licensing Agreement with Vifor Pharma for the Commercialization of Sparsentan in Europe, Australia and New Zealand

- During the third quarter of 2021, the Company and Vifor Pharma announced a joint collaboration and licensing agreement for the commercialization of sparsentan in Europe, Australia and New Zealand. Under the terms of the agreement, Vifor Pharma receives exclusive commercialization rights for sparsentan in Europe, Australia and New Zealand. Travere

received an upfront payment of $55.0 million and will be eligible for up to $135.0 million in payments tied to the achievement of certain regulatory and market access related milestones. Vifor Pharma will also make further payments in the form of sales milestones, and tiered double-digit royalties on net sales of sparsentan in Europe, Australia and New Zealand up to 40 percent at the high end of the royalty range.

Conference Call Information

Travere Therapeutics will host a conference call and webcast today, Thursday, October 28, 2021 at 4:30 p.m. ET to discuss company updates as well as third quarter 2021 financial results. To participate in the conference call, dial +1 (855) 219-9219 (U.S.) or +1 (315) 625-6891 (International), confirmation code 5068414 shortly before 4:30 p.m. ET. The webcast can be accessed at travere.com, in the Events and Presentations section of the Investors & Media page, and will be archived for at least 30 days. A replay of the call will be available from 7:30 p.m. ET, October 28, 2021 to 7:30 p.m. ET, November 4, 2021. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 5068414.

Use of Non-GAAP Financial Measures

To supplement Travere's financial results and guidance presented in accordance with U.S. generally accepted accounting principles (GAAP), the Company uses certain non-GAAP adjusted financial measures in this press release and the accompanying tables. The Company believes that these non-GAAP financial measures are helpful in understanding its past financial performance and potential future results. They are not meant to be considered in isolation or as a substitute for comparable GAAP measures and should be read in conjunction with the consolidated financial statements prepared in accordance with GAAP. Travere's management regularly uses these supplemental non-GAAP financial measures internally to understand, manage and evaluate its business and make operating decisions. In addition, Travere believes that the use of these non-GAAP measures enhances the ability of investors to compare its results from period to period and allows for greater transparency with respect to key financial metrics the Company uses in making operating decisions.

Investors should note that these non-GAAP financial measures are not prepared under any comprehensive set of accounting rules or principles and do not reflect all of the amounts associated with the Company's results of operations as determined in accordance with GAAP. Investors should also note that these non-GAAP financial measures have no standardized meaning prescribed by GAAP and, therefore, have limits in their usefulness to investors. In addition, from time to time in the future the Company may exclude other items, or cease to exclude items that it has historically excluded, for purposes of its non-GAAP financial measures; because of the non-standardized definitions, the non-GAAP financial measures as used by the Company in this press release and the accompanying tables may be calculated differently from, and therefore may not be directly comparable to, similarly titled measures used by the Company's competitors and other companies.

As used in this press release, (i) the historical non-GAAP net income (loss) measures exclude from GAAP net income (loss), as applicable, stock-based compensation expense, amortization and depreciation expense, revaluation of acquisition related contingent consideration and income tax; (ii) the historical non-GAAP SG&A expense measures exclude from GAAP SG&A expenses, as applicable, stock-based compensation expense, and amortization and depreciation expense; (iii) the historical non-GAAP R&D expense measures exclude from GAAP R&D expenses, as applicable, stock-based compensation expense, and depreciation and amortization expense.

About Travere Therapeutics

At Travere Therapeutics we are in rare for life. We are a biopharmaceutical company that comes together every day to help patients, families and caregivers of all backgrounds as they navigate life with a rare disease. On this path, we know the need for treatment options is urgent – that is why our global team works with the rare disease community to identify, develop and deliver life-changing therapies. In pursuit of this mission, we continuously seek to understand the diverse perspectives of rare patients and to courageously forge new paths to make a difference in their lives and provide hope – today and tomorrow. For more information, visit travere.com.

Forward-Looking Statements

This press release contains "forward-looking statements" as that term is defined in the Private Securities Litigation Reform Act of 1995. Without limiting the foregoing, these statements are often identified by the words "may", "might", "believes", "thinks", "anticipates", "plans", "expects", "intends" or similar expressions. In addition, expressions of our strategies, intentions or plans are also forward-looking statements. Such forward-looking statements include, but are not limited to, references to: expected sparsentan regulatory submissions in 2022, including the timing for the planned IgAN accelerated approval submission, the ability to submit for accelerated approval in FSGS, pending additional supportive eGFR data, as well as expectations regarding submitting a joint marketing authorization application in Europe for both FSGS and IgAN; the Company's expectations for multiple potential launches of sparsentan, if approved; references to the efficacy, safety and tolerability profile of sparsentan based on the preliminary data from the DUPLEX and PROTECT Studies' interim analyses and expectations that additional eGFR data from the DUPLEX Study will support an accelerated approval submission; the Company's potential future receipt of regulatory, market-access and sales based milestones and royalties under the joint collaboration and licensing agreement with Vifor; the Company's expectations around timelines for preliminary data from the ongoing Phase 1/2 study of pegtibatinase in HCU; and references to the potential for pegtibatinase, if approved, to become the first disease modifying therapy for people living with classical HCU. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties associated with the regulatory review and approval process, including the Subpart H accelerated approval pathway in the United States and the conditional marketing authorization (CMA) pathway in the European Union, as well as risks and uncertainties associated with the Company's business and finances in general, success of its commercial products and risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with market acceptance of its commercial products including efficacy, safety, price, reimbursement and benefit over competing therapies. The risks and uncertainties the Company faces with respect to its preclinical and clinical stage pipeline include risk that the Company's clinical candidates will not be found to be safe or effective and that current clinical trials will not proceed as planned. Specifically, the Company faces the risk that the Phase 3 DUPLEX Study of sparsentan in FSGS will not demonstrate that sparsentan is safe or effective or serve as a basis for accelerated approval of sparsentan as planned; risk that the Phase 3 PROTECT Study of sparsentan in IgAN will not demonstrate that sparsentan is safe or effective or serve as the basis for accelerated approval of sparsentan as planned; and risk that sparsentan will not be approved for efficacy, safety, regulatory or other reasons, and for each of the Company's programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing or planned

clinical trials may not succeed or may be delayed for safety, regulatory or other reasons. There is no guarantee that the FDA will grant accelerated approval of sparsentan for IgAN or FSGS or that sparsentan will be approved at all. There is also no guarantee that the results from the ongoing clinical study of pegtibatinase will be positive. The Company faces risk that it will be unable to raise additional funding that may be required to complete development of any or all of its product candidates; risk relating to the Company's dependence on contractors for clinical drug supply and commercial manufacturing; uncertainties relating to patent protection and exclusivity periods and intellectual property rights of third parties; risks associated with regulatory interactions; risks and uncertainties relating to competitive products, including current and potential future generic competition with certain of the Company's products, and technological changes that may limit demand for the Company's products. The Company faces additional risks associated with the potential impacts the COVID-19 pandemic may have on its business, including, but not limited to (i) the Company's ability to continue its ongoing development activities and clinical trials, (ii) the timing of such clinical trials and the release of data from those trials, (iii) the Company's and its suppliers' ability to successfully manufacture its commercial products and product candidates, and (iv) the market for and sales of its commercial products. You are cautioned not to place undue reliance on these forward-looking statements as there are important factors that could cause actual results to differ materially from those in forward-looking statements, many of which are beyond our control. The Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise. Investors are referred to the full discussion of risks and uncertainties as included in the Company's most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

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TRAVERE THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share amounts)

	September 30, 2021	December 31, 2020
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 150,327	$ 84,772
Available-for-sale debt securities, at fair value (amortized cost $400,866, allowance for credit losses of $0 as of September 30, 2021; amortized cost $276,111, allowance for credit losses of $0 as of December 31, 2020)	400,857	276,817
Accounts receivable, net	13,370	15,925
Inventory, net	6,616	7,608
Prepaid expenses and other current assets	7,504	8,143
Tax receivable	405	17,142
Total current assets	579,079	410,407
Property and equipment, net	11,513	9,418
Other non-current assets	34,871	33,489
Intangible assets, net	148,676	153,189
Goodwill	936	936
Total assets	$ 775,075	$ 607,439
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 5,830	$ 12,133
Accrued expenses	63,308	56,793
Other current liabilities	10,056	6,334
Deferred revenue, current portion	16,069	—
Business combination-related contingent consideration, current portion	17,900	17,400
Total current liabilities	113,163	92,660
Convertible debt	223,696	215,339
Other non-current liabilities	43,262	40,527
Deferred revenue, less current portion	24,084	—
Business combination-related contingent consideration, less current portion	63,500	47,700
Total liabilities	467,705	396,226
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2021 and December 31, 2020	—	—

Common stock $0.0001 par value; 200,000,000 shares authorized; 61,018,229 and 52,248,431 issued and outstanding as of September 30, 2021 and December 31, 2020, respectively	6	5
Additional paid-in capital	1,022,282	797,985
Accumulated deficit	(714,393)	(585,875)
Accumulated other comprehensive loss	(525)	(902)
Total stockholders' equity	307,370	211,213
Total liabilities and stockholders' equity	$ 775,075	$ 607,439

Note: Certain adjustments / reclassifications have been made to prior periods to conform to current year presentation.

TRAVERE THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
	(unaudited)			
Net product sales:				
Thiola/Thiola EC	$ 29,821	$ 28,227	$ 84,907	$ 80,572
Bile acid products	24,353	22,912	71,291	66,766
Total net product sales	54,174	51,139	156,198	147,338
License revenue	14,043	—	14,043	—
Total revenue	68,217	51,139	170,241	147,338
Operating expenses:				
Cost of goods sold	1,592	1,189	4,888	4,054
Research and development	48,407	32,349	148,160	93,387
Selling, general and administrative	36,065	31,951	107,808	100,061
Change in fair value of contingent consideration	13,864	5,085	23,960	7,448
Total operating expenses	99,928	70,574	284,816	204,950
Operating loss	(31,711)	(19,435)	(114,575)	(57,612)
Other income (expenses), net:				
Other income (expense), net	654	553	(223)	788
Interest income	360	1,123	1,757	4,414
Interest expense	(4,899)	(4,767)	(15,072)	(14,287)
Total other expense, net	(3,885)	(3,091)	(13,538)	(9,085)
Loss before income taxes	(35,596)	(22,526)	(128,113)	(66,697)
Income tax (expense) benefit	(43)	(23)	(405)	18,888
Net loss	$ (35,639)	$ (22,549)	$ (128,518)	$ (47,809)
Per share data:				
Basic and diluted net loss per common share	$ (0.59)	$ (0.44)	$ (2.17)	$ (1.03)
Basic and diluted weighted average common shares outstanding	60,803,045	50,929,575	59,230,881	46,289,103
Weighted average common shares outstanding, diluted	60,803,045	50,929,575	59,230,881	46,289,103

Note: Certain adjustments / reclassifications have been made to prior periods to conform to current year presentation.

TRAVERE THERAPEUTICS, INC.
RECONCILIATION OF GAAP REPORTED TO NON-GAAP ADJUSTED INFORMATION
(in thousands, except share and per share data)
(*unaudited*)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
GAAP operating loss	$ (31,711)	$ (19,435)	$ (114,575)	$ (57,612)
R&D operating expense	(48,407)	(32,349)	(148,160)	(93,387)
Stock compensation	2,630	2,510	8,477	6,968
Amortization & depreciation	614	292	1,188	870
Subtotal non-GAAP items	3,244	2,802	9,665	7,838
Non-GAAP R&D expense	(45,163)	(29,547)	(138,495)	(85,549)
SG&A operating expense	(36,065)	(31,951)	(107,808)	(100,061)
Stock compensation	4,356	2,888	13,713	10,294
Amortization & depreciation	6,250	6,168	18,369	17,076
Subtotal non-GAAP items	10,606	9,056	32,082	27,370
Non-GAAP SG&A expense	(25,459)	(22,895)	(75,726)	(72,691)
Change in fair value of contingent consideration	13,864	5,085	23,960	7,448
Subtotal non-GAAP items	27,714	16,943	65,707	42,656
Non-GAAP operating loss	**$ (3,997)**	**$ (2,492)**	**$ (48,868)**	**$ (14,956)**
GAAP net income (loss)	**$ (35,639)**	**$ (22,549)**	**$ (128,518)**	**$ (47,809)**
Non-GAAP operating loss adjustments	27,714	16,943	65,707	42,656
Income tax provision (benefit)	43	23	405	(18,888)
Non-GAAP net loss	**$ (7,882)**	**$ (5,583)**	**$ (62,406)**	**$ (24,041)**
Per share data:				
Basic and diluted net loss per common share	$ (0.13)	$ (0.11)	$ (1.05)	$ (0.52)
Basic and diluted weighted average common shares outstanding	60,803,045	50,929,575	59,230,881	46,289,103

Note: Certain adjustments / reclassifications have been made to prior periods to conform to current year presentation.

TRAVERE THERAPEUTICS

Source: Travere Therapeutics, Inc.