Travere Therapeutics Reports Second Quarter 2021 Financial Results

July 29, 2021

Company expects to submit an application for conditional marketing authorization of sparsentan for FSGS in Europe before year-end 2021

Pivotal PROTECT Study of sparsentan in IgA nephropathy on track to report topline data from interim 36-week proteinuria endpoint in August 2021

Net product sales of $54.6 million for the second quarter of 2021

SAN DIEGO, July 29, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today reported its second quarter 2021 financial results and provided a corporate update.

- Following interactions with the European Medicines Agency (EMA) during the second quarter of 2021, the Company expects to submit an application for conditional marketing authorization (CMA) of sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS) in Europe before year-end 2021
- The Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN) completed patient enrollment in the second quarter of 2021; topline data from the 36-week interim proteinuria analysis are on track for August 2021
- Net product sales for the second quarter 2021 were $54.6 million, compared to $48.4 million for the same period in 2020
- Cash, cash equivalents and marketable securities, as of June 30, 2021, totaled $522.8 million

“We remain steadfast in our commitment to advancing toward our goal of delivering sparsentan as a potential new treatment standard for people living with FSGS and IgAN,” said Eric Dube, Ph.D., chief executive officer of Travere Therapeutics. “We are encouraged by recent interactions with EMA, and we remain on-track to submit an application for conditional marketing authorization for FSGS in Europe later this year. As previously announced, we’ve adjusted our U.S. timelines for FSGS and look forward to our upcoming Type A meeting with the FDA to further our collaborative discussions on the potential to stay on an accelerated approval pathway. Next month, we are looking forward to the upcoming topline results from our Phase 3 PROTECT Study of sparsentan in IgA nephropathy, which if successful, would contribute to the growing body of evidence for sparsentan in rare kidney disorders, and potentially support submissions for accelerated approval in the U.S. and conditional marketing authorization in Europe.”

Quarter Ended June 30, 2021

Net product sales for the second quarter of 2021 were $54.6 million, compared to $48.4 million for the same period in 2020. For the six months ended June 30, 2021, net product sales were $102.0 million, compared to $96.2 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 second quarter of 2021 were $51.8 million, compared to $30.8 million for the same period in 2020. For the six months ended June 30, 2021, R&D expenses were $99.8 million, compared to $61.0 million for the same period in 2020. The difference is largely attributable to the fully enrolled, ongoing pivotal DUPLEX and PROTECT studies of sparsentan, as well the continued development of the pegtibatinase program in classical homocystinuria (HCU). On a non-GAAP adjusted basis, R&D expenses were $48.7 million for the second quarter of 2021, compared to $28.2 million for the same period in 2020.

Selling, general and administrative (SG&A) expenses for the second quarter of 2021 were $35.0 million, compared to $35.0 million for the same period in 2020. For the six months ended June 30, 2021, SG&A expenses were $71.7 million, compared to $68.1 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 $24.0 million for the second quarter of 2021, compared to $25.8 million for the same period in 2020.

Total other expense, net, for the second quarter of 2021 was $3.6 million, compared to $2.9 million for the same period in 2020. The difference is largely attributable to a reduction in interest income.

Net loss for the second quarter of 2021 was $39.0 million, or $0.64 per basic share, compared to a net loss of $26.1 million, or $0.58 per basic share for the same period in 2020. For the six months ended June 30, 2021, net loss was $92.9 million, compared to $25.3 million for the same period in 2020. On a non-GAAP adjusted basis, net loss for the second quarter of 2021 was $23.3 million, or $0.39 per basic share, compared to a net loss of $9.9 million, or $0.22 per basic share for the same period in 2020.

As of June 30, 2021, the Company had cash, cash equivalents and marketable securities of $522.8 million.

Program Updates

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 from the interim analysis suggest

that at the time of the interim assessment, sparsentan had been generally well-tolerated and shown a comparable safety profile to irbesartan. The DUPLEX Study is fully enrolled and is scheduled to continue as planned on a blinded basis to assess the confirmatory estimated glomerular filtration rate (eGFR) endpoint after 108 weeks of treatment. Topline results from the confirmatory endpoint are expected in the first half of 2023.

- During the Company's pre-NDA meeting with the U.S. Food and Drug Administration (FDA) in the second quarter of 2021, the FDA indicated that it may be possible to submit an accelerated approval application for sparsentan in FSGS after additional data accrue in the ongoing DUPLEX Study. The Company has scheduled a Type A meeting during the third quarter of 2021 to discuss the potential to provide additional eGFR data from the DUPLEX Study in the first half of 2022, in an effort to enable an accelerated approval submission next year.

- During the second quarter of 2021, the Company conducted Marketing Authorization Application (MAA) pre-submission interactions with assigned rapporteurs and co-rapporteurs from the EMA. Following these interactions, the Company is planning to submit a CMA application for sparsentan for the treatment of FSGS in Europe before year-end 2021.

Sparsentan - IgAN

- During the second quarter of 2021, the pivotal Phase 3 PROTECT Study of sparsentan in IgAN completed patient enrollment. The PROTECT Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial evaluating the safety and efficacy of sparsentan in approximately 380 patients with IgAN. The PROTECT Study protocol provides for an unblinded interim analysis of at least 280 patients to be performed after 36 weeks of treatment to evaluate the primary efficacy endpoint – the change in proteinuria (urine protein-to-creatinine ratio) at Week 36 from baseline. The interim assessment of the PROTECT Study is designed to support potential submissions under the Subpart H pathway for accelerated approval in the United States, and potential Conditional Marketing Authorization in Europe. Secondary efficacy endpoints include the rate of change in eGFR following the initiation of randomized treatment over 58-week and 110-week periods, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment in approximately 380 patients. Topline efficacy data from the 36-week interim proteinuria endpoint analysis are anticipated in August 2021.

Pegtibatinase (TVT-058)

- During the second quarter of 2021, the Company completed enrollment in the highest currently planned dosing cohort in the ongoing Phase 1/2 dose escalation study to assess the safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects of pegtibatinase in patients with classical HCU. The Company anticipates preliminary data from the Phase 1/2 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.

Conference Call Information

Travere Therapeutics will host a conference call and webcast today, Thursday, July 29, 2021 at 4:30 p.m. ET to discuss company updates as well as second 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 9790904 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, July 29, 2021 to 7:30 p.m. ET, August 5, 2021. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 9790904.

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: the Company's current plan regarding, and expectations around the timeline for, submitting an application for conditional marketing authorization (CMA) of sparsentan for FSGS in Europe; expectations around the planned Type A meeting with the FDA and the potential to stay on an accelerated approval pathway for FSGS in the U.S. and submit an application for accelerated approval in 2022; the Company's current expectations around timelines for top-line data from the proteinuria endpoint in the PROTECT study and the confirmatory endpoint in the DUPLEX Study; references to the potential outcome of the PROTECT study, including the potential for the results to support an accelerated approval submission in IgAN; references to the Company's goal of delivering sparsentan as a potential new treatment standard for people living with FSGS and IgAN; the Company's current 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 Europe Union, including the risk that the FDA or EMA could disagree with the Company's submission of an NDA under Subpart H for accelerated approval, or a Marketing Approval Application ("MAA") under the CMA pathway, as well as risks and uncertainties associated with the Company's business and finances in general, success of its commercial products as well as 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 and the Company will be able to align on a pathway for a potential accelerated approval submission for sparsentan in FSGS; that the FDA will grant accelerated approval of sparsentan for FSGS or that sparsentan will be approved at all. There is also no guarantee that the results from the PROTECT Study in IgAN will be positive, or even if positive, will support an accelerated approval submission in the U.S. or a submission under the CMA pathway in Europe or 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 potential 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.

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

	June 30, 2021	December 31, 2020
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 83,288	$ 84,772
Available-for-sale debt securities, at fair value (amortized cost $439,411, allowance for credit losses of $0 as of June 30, 2021; amortized cost $276,111, allowance for credit losses of $0 as of December 31, 2020)	439,502	276,817
Accounts receivable, net	11,860	15,925

Inventory, net	7,409	7,608
Prepaid expenses and other current assets	7,339	8,143
Tax receivable	400	17,142
Total current assets	549,798	410,407
Property and equipment, net	11,720	9,418
Other non-current assets	34,361	33,489
Intangible assets, net	149,951	153,189
Goodwill	936	936
Total assets	$ 746,766	$ 607,439
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 8,343	$ 12,133
Accrued expenses	62,465	56,793
Other current liabilities	8,869	6,334
Business combination-related contingent consideration, current portion	17,300	17,400
Total current liabilities	96,977	92,660
Convertible debt	220,861	215,339
Other non-current liabilities	43,725	40,527
Business combination-related contingent consideration, less current portion	52,900	47,700
Total liabilities	414,463	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; 60,710,876 and 52,248,431 issued and outstanding as of June 30, 2021 and December 31, 2020, respectively	6	5
Additional paid-in capital	1,011,692	797,985
Accumulated deficit	(678,754)	(585,875)
Accumulated other comprehensive loss	(641)	(902)
Total stockholders' equity	332,303	211,213
Total liabilities and stockholders' equity	$ 746,766	$ 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 June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
	(unaudited)			
Net product sales:				
Thiola/Thiola EC	$ 29,643	$ 26,857	$ 55,086	$ 52,345
Bile acid products	24,974	21,573	46,938	43,854
Total net product sales	54,617	48,430	102,024	96,199
Operating expenses:				
Cost of goods sold	1,651	1,494	3,296	2,864
Research and development	51,807	30,790	99,753	61,038
Selling, general and administrative	34,965	34,971	71,743	68,110
Change in fair value of contingent consideration	1,509	4,286	10,096	2,363
Total operating expenses	89,932	71,541	184,888	134,375
Operating loss	(35,315)	(23,111)	(82,864)	(38,176)
Other income (expenses), net:				
Other income (expense), net	216	426	(877)	235

Interest income	988	1,316	1,397	3,291
Interest expense	(4,852)	(4,634)	(10,173)	(9,521)
Total other expense, net	(3,648)	(2,892)	(9,653)	(5,995)
Loss before income taxes	(38,963)	(26,003)	(92,517)	(44,171)
Income tax (expense) benefit	(49)	(65)	(362)	18,911
Net loss	$ (39,012)	$ (26,068)	$ (92,879)	$ (25,260)
Per share data:				
Basic and diluted net loss per common share	$ (0.64)	$ (0.58)	$ (1.59)	$ (0.57)
Basic and diluted weighted average common shares outstanding	60,571,259	44,763,843	58,431,770	43,943,370
Weighted average common shares outstanding, diluted	60,571,259	44,763,843	58,431,770	43,943,370

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 June 30,		Six Months Ended June 30,	
	2021	**2020**	**2021**	**2020**
GAAP operating loss	$ (35,315)	$ (23,111)	$ (82,864)	$ (38,176)
R&D operating expense	(51,807)	(30,790)	(99,753)	(61,038)
Stock compensation	2,845	2,332	5,847	4,458
Amortization & depreciation	288	289	574	578
Subtotal non-GAAP items	3,133	2,621	6,421	5,036
Non-GAAP R&D expense	(48,674)	(28,169)	(93,332)	(56,002)
SG&A operating expense	(34,965)	(34,971)	(71,743)	(68,110)
Stock compensation	4,665	3,622	9,357	7,406
Amortization & depreciation	6,330	5,542	12,119	10,908
Subtotal non-GAAP items	10,995	9,164	21,476	18,314
Non-GAAP SG&A expense	(23,970)	(25,807)	(50,267)	(49,796)
Change in fair value of contingent consideration	1,509	4,286	10,096	2,363
Subtotal non-GAAP items	15,637	16,071	37,993	25,713
Non-GAAP operating loss	**$ (19,678)**	**$ (7,040)**	**$ (44,871)**	**$ (12,463)**
GAAP net income (loss)	**$ (39,012)**	**$ (26,068)**	**$ (92,879)**	**$ (25,260)**
Non-GAAP operating loss adjustments	15,637	16,071	37,993	25,713
Income tax provision (benefit)	49	65	362	(18,911)
Non-GAAP net loss	**$ (23,326)**	**$ (9,932)**	**$ (54,524)**	**$ (18,458)**
Per share data:				
Basic and diluted net loss per common share	$ (0.39)	$ (0.22)	$ (0.93)	$ (0.42)
Basic and diluted weighted average common shares outstanding	60,571,259	44,763,843	58,431,770	43,943,370

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

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