Travere Therapeutics Reports First Quarter 2021 Financial Results

May 6, 2021

Pivotal PROTECT Study of sparsentan in IgA nephropathy nears completion of patient enrollment; topline proteinuria data expected 3Q21

Net product sales of $47.4 million for the first quarter of 2021

SAN DIEGO, May 06, 2021 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today reported its first quarter 2021 financial results and provided a corporate update.

- Following achievement of the pre-specified interim FSGS partial remission of proteinuria endpoint (FPRE) in the ongoing Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS), the Company is engaging with regulators in pursuit of submissions for accelerated approval
- The Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN) is nearing completion of patient enrollment and remains on-track to report topline data from the 36-week proteinuria analysis in the third quarter of 2021
- Net product sales for the first quarter 2021 were $47.4 million, compared to $47.8 million for the same period in 2020
- Cash, cash equivalents and marketable securities, as of March 31, 2021, totaled $520.9 million

"I am pleased with our start to 2021. Our organization is fully aligned and executing on the priorities that will enable us to develop our pipeline and ultimately deliver new treatments to people living with rare diseases," said Eric Dube, Ph.D., chief executive officer of Travere Therapeutics. "To begin this year, our Phase 3 DUPLEX Study of sparsentan achieved its interim proteinuria endpoint. We are in the process of engaging with the FDA and EMA in pursuit of the accelerated approval pathway for FSGS, and we look forward to providing a regulatory update in the first half of the year as previously announced. During the first quarter, we saw strong patient enrollment in our pivotal PROTECT Study of sparsentan in IgA nephropathy. This has positioned us to randomize the last patient in the study in the near future and report topline results from the interim proteinuria analysis in the third quarter of this year. Finally, we continued to strengthen our commercialization capabilities. This led to reaching new patients with our approved products despite the ongoing pandemic and has further prepared our organization to be successful with future potential launches from our pipeline."

First Quarter 2021 Financial Results

Net product sales for the first quarter of 2021 were $47.4 million, compared to $47.8 million for the same period in 2020. The company estimated a favorable impact of approximately $1.5 million to $2.0 million in the first quarter of 2020 as a result of increased demand at the onset of the COVID-19 pandemic. For the full year 2021, the Company continues to anticipate mid-single-digit percentage growth in net product sales compared to 2020.

Research and development (R&D) expenses for the first quarter of 2021 were $47.9 million, compared to $30.2 million for the same period in 2020. The difference is largely attributable to increased patient enrollment in the ongoing pivotal DUPLEX and PROTECT studies of sparsentan, as well the addition of the pegtibatinase (TVT-058) program in classical homocystinuria (HCU). On a non-GAAP adjusted basis, R&D expenses were $44.7 million for the first quarter of 2021, compared to $27.8 million for the same period in 2020.

Selling, general and administrative (SG&A) expenses for the first quarter of 2021 were $36.8 million, compared to $33.1 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 $26.3 million for the first quarter of 2021, compared to $24.0 million for the same period in 2020.

Total other expense, net, for the first quarter of 2021 was $6.0 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 first quarter of 2021 was $53.9 million, or $0.96 per basic share, compared to a net income of $0.8 million, or $0.02 per basic share for the same period in 2020. On a non-GAAP adjusted basis, net loss for the first quarter of 2021 was $31.2 million, or $0.55 per basic share, compared to a net loss of $8.5 million, or $0.20 per basic share for the same period in 2020.

As of March 31, 2021, the Company had cash, cash equivalents and marketable securities of $520.9 million. This includes net proceeds of approximately $189 million from a common stock offering completed in February 2021.

Program Updates

Sparsentan

- In February 2021, the Company announced that the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in FSGS achieved its pre-specified interim FPRE endpoint 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 to date in the study, sparsentan has been generally well-tolerated and has shown a comparable safety profile to irbesartan. Based on the data from the interim analysis, the Company is continuing its engagement with regulators in the first half of 2021 to discuss the ongoing study and the pursuit of an NDA submission under the Subpart H accelerated approval pathway in the U.S., as well as an

application for CMA consideration in Europe. 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.

- The PROTECT Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled pivotal Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 380 patients with IgAN is nearing completion of patient enrollment. The PROTECT Study protocol provides for an unblinded 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. Successful achievement of the proteinuria endpoint is expected to support submission of an NDA under the Subpart H accelerated approval pathway in the U.S., as well as an application for CMA consideration 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 the third quarter of 2021.

Pegtibatinase (TVT-058)

- During the first quarter of 2021, the International Nonproprietary Names (INN) Expert Group published the nonproprietary name "pegtibatinase" for TVT-058, the Company's novel investigational human enzyme replacement therapy for the treatment of classical HCU.

- The Phase 1/2 dose escalation study to assess the safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects of pegtibatinase in patients with classical HCU continues to advance. The Company anticipates preliminary data from the Phase 1/2 study 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, May 6, 2021 at 4:30 p.m. ET to discuss company updates as well as first quarter 2021 financial results. To participate in the conference call, dial +1-800-773-2954 (U.S.) or +1-847-413-3731 (International), confirmation code 50155370 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, May 6, 2021 to 7:30 p.m. ET, May 13, 2021. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 50155370.

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 plans for regulatory submissions for sparsentan under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe; the Company's current expectations around the timeline for providing a regulatory update around the sparsentan FSGS program; 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; the Company's current expectations around timelines for preliminary data from the ongoing Phase 1/2 study of OT-58 in HCU; the potential for OT-58 to become the first disease modifying therapy for people living with HCU; and the Company's expectations regarding 2021 growth of the Company's net product sales compared to 2020. 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 planned 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 will accept for filing the Company's planned NDA for sparsentan for FSGS under the Subpart H approval pathway, that the FDA will grant accelerated approval of sparsentan for FSGS or that sparsentan will be approved at all. 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)

	March 31, 2021	December 31, 2020
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 148,232	$ 84,772
Available-for-sale debt securities, at fair value (amortized cost $372,396, allowance for credit losses of $0 as of March 31, 2021; amortized cost $276,111, allowance for credit losses of $0 as of December 31, 2020)	372,639	276,817
Accounts receivable, net	13,066	15,925
Inventory, net	7,183	7,608
Prepaid expenses and other current assets	6,747	8,143
Tax receivable	17,173	17,142
Total current assets	565,040	410,407
Property and equipment, net	11,697	9,418
Other non-current assets	35,030	33,489
Intangible assets, net	151,342	153,189
Goodwill	936	936
Total assets	$ 764,045	$ 607,439
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 15,008	$ 12,133
Accrued expenses	46,467	56,793

Other current liabilities	7,338	6,334
Business combination-related contingent consideration, current portion	16,200	17,400
Total current liabilities	85,013	92,660
Convertible debt	218,076	215,339
Other non-current liabilities	43,167	40,527
Business combination-related contingent consideration, less current portion	55,100	47,700
Total liabilities	401,356	396,226
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2021 and December 31, 2020	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 60,435,730 and 52,248,431 issued and outstanding as of March 31, 2021 and December 31, 2020, respectively	6	5
Additional paid-in capital	1,002,687	797,985
Accumulated deficit	(639,742)	(585,875)
Accumulated other comprehensive income	(262)	(902)
Total stockholders' equity	362,689	211,213
Total liabilities and stockholders' equity	$ 764,045	$ 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 March 31,	
	2021	**2020**
	(unaudited)	
Net product sales:		
Thiola/Thiola EC	$ 25,443	$ 25,488
Bile acid products	21,964	22,281
Total net product sales	47,407	47,769
Operating expenses:		
Cost of goods sold	1,645	1,370
Research and development	47,946	30,249
Selling, general and administrative	36,778	33,139
Change in fair value of contingent consideration	8,587	(1,922)
Total operating expenses	94,956	62,836
Operating loss	(47,549)	(15,067)
Other income (expenses), net:		
Other income (expense), net	(1,093)	(190)
Interest income	409	1,976
Interest expense	(5,321)	(4,887)
Total other expense, net	(6,005)	(3,101)
Loss before income taxes	(53,554)	(18,168)
Income tax (expense) benefit	(313)	18,976
Net income (loss)	$ (53,867)	$ 808
Per share data:		
Net income (loss) per common share, basic	$ (0.96)	$ 0.02
Net income (loss) per common share, diluted	$ (0.96)	$ 0.02
Weighted average common shares outstanding, basic	56,268,508	43,122,897
Weighted average common shares outstanding, diluted	56,268,508	43,592,499

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 March 31,	
	2021	**2020**
GAAP operating loss	$ (47,549)	$ (15,067)
R&D operating expense	(47,946)	(30,249)
Stock compensation	3,002	2,126
Amortization & depreciation	286	289
Subtotal non-GAAP items	3,288	2,415
Non-GAAP R&D expense	(44,658)	(27,834)
SG&A operating expense	(36,778)	(33,139)
Stock compensation	4,692	3,784
Amortization & depreciation	5,789	5,366
Subtotal non-GAAP items	10,481	9,150
Non-GAAP SG&A expense	(26,297)	(23,989)
Change in fair value of contingent consideration	8,587	(1,922)
Subtotal non-GAAP items	22,356	9,643
Non-GAAP operating loss	**$ (25,193)**	**$ (5,424)**
GAAP net income (loss)	**$ (53,867)**	**$ 808**
Non-GAAP operating loss adjustments	22,356	9,643
Income tax provision (benefit)	313	(18,976)
Non-GAAP net loss	**$ (31,198)**	**$ (8,525)**
Per share data:		
Basic and diluted net loss per common share	$ (0.55)	$ (0.20)
Basic and diluted weighted average common shares outstanding	56,268,508	43,122,897

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

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