Travere Therapeutics Reports First Quarter 2022 Financial Results

May 5, 2022

Submitted New Drug Application (NDA) for sparsentan in IgA nephropathy in March 2022

Results from Phase 1/2 COMPOSE Study of pegtibatinase in classical homocystinuria (HCU) presented at the Society for Inherited Metabolic Disorders (SIMD) 43rd Annual Meeting

Refinanced $207 million of 2025 convertible notes through offering of $316 million convertible senior notes due 2029

SAN DIEGO, May 05, 2022 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today reported its first quarter 2022 financial results and provided a corporate update.

- New Drug Application (NDA) for accelerated approval of sparsentan in IgA nephropathy (IgAN) submitted to the U.S. Food and Drug Administration (FDA) in March 2022; notice regarding acceptance expected May 2022
- Previously reported positive topline results from the Phase 1/2 COMPOSE Study of pegtibatinase in classical homocystinuria (HCU) presented at the Society for Inherited Metabolic Disorders (SIMD) Annual Meeting in April 2022
- Total revenue for the first quarter 2022 was $48.5 million, consisting of $46.4 million in net product sales and $2.0 million in licensing and collaboration revenue
- Cash, cash equivalents and marketable securities, as of March 31, 2022, totaled $603.4 million

“To begin the year, we continued to execute towards our goal of making sparsentan a new treatment standard for rare kidney disorders, if approved,” said Eric Dube, Ph.D., president and chief executive officer of Travere Therapeutics. “In March, we submitted our NDA for accelerated approval of sparsentan for IgA nephropathy and we remain on track for our planned regulatory engagements for FSGS. We continue to make significant headway in our preparations for the potential commercial launch of sparsentan in IgA nephropathy which could occur as early as the end of this year. Together with the continued advancement of our pegtibatinase program and the strengthening of our balance sheet during the quarter, we are well-positioned for continued execution throughout the balance of 2022.”

Quarter Ended March 31, 2022

Net product sales for the first quarter of 2022 were $46.4 million, compared to $47.4 million for the same period in 2021. The difference is largely attributable to a decrease in Thiola sales partially offset by an increase in sales for the Company’s bile acid products.

Research and development (R&D) expenses for the first quarter of 2022 were $56.6 million, compared to $47.9 million for the same period in 2021. 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. On a non-GAAP adjusted basis, R&D expenses were $53.2 million for the first quarter of 2022, compared to $44.7 million for the same period in 2021.

Selling, general and administrative (SG&A) expenses for the first quarter of 2022 were $46.8 million, compared to $36.8 million for the same period in 2021. The difference is largely attributable to increased headcount as a result of the Company’s operational growth, and commercial launch preparations. On a non-GAAP adjusted basis, SG&A expenses were $35.0 million for the first quarter of 2022, compared to $26.3 million for the same period in 2021.

Total other expense, net, for the first quarter of 2022 was $9.8 million, compared to $6.0 million for the same period in 2021. The difference is largely attributable to changes in interest expense and a loss on early extinguishment of debt related to the Company’s convertible note transactions effected in March 2022.

Net loss for the first quarter of 2022 was $76.0 million, or $1.20 per basic share, compared to a net loss of $53.9 million, or $0.96 per basic share for the same period in 2021. On a non-GAAP adjusted basis, net loss for the first quarter of 2022 was $51.6 million, or $0.82 per basic share, compared to a net loss of $31.2 million, or $0.55 per basic share for the same period in 2021.

As of March 31, 2022, the Company had cash, cash equivalents and marketable securities of $603.4 million. This includes net cash proceeds of approximately $95 million from the Company’s convertible note transactions in March 2022 in which approximately $316 million in 2.25% convertible notes due 2029 were issued and approximately $207 million of $276 million outstanding 2.50% convertible notes due 2025 were repurchased.

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.

- In March 2022, the Company submitted an NDA to the FDA under Subpart H for accelerated approval of sparsentan for the treatment IgAN. The Company expects to receive notice regarding the acceptance of the NDA, as well as the timeline for NDA review, from the FDA in May 2022.

Sparsentan - FSGS

- In February 2021, the Company announced that the ongoing pivotal Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (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 analysis are expected in the first half of 2023.

- The Company remains on-track to provide additional eGFR data from the ongoing DUPLEX Study to the FDA and to provide an update in mid-2022 on a potential NDA submission for accelerated approval of sparsentan for FSGS in the U.S., and with its partner Vifor Pharma, a combined IgAN and FSGS Marketing Authorisation Application (MAA) in Europe.

Pegtibatinase (TVT-058)

- In December 2021, the Company reported positive topline results from the ongoing Phase 1/2 COMPOSE Study of pegtibatinase, a novel investigational enzyme replacement therapy with the potential to become the first disease modifying therapy for people living with HCU. In the highest dose cohort to date evaluating 1.5mg/kg of pegtibatinase twice weekly, treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine (tHcy) through 12 weeks of treatment, including a 55.1 percent mean relative reduction in tHcy from baseline as well as maintenance of tHcy below a clinically meaningful threshold of 100 µmol. To date in the study, pegtibatinase has been generally well-tolerated.

- In April 2022, the Company presented detailed study results from the ongoing Phase 1/2 COMPOSE Study at the Society for Inherited Metabolic Disorders (SIMD) 43rd Annual Meeting.

- The Company is engaging with regulators to establish next steps for a pivotal development program to ultimately support potential approvals of pegtibatinase for the treatment of HCU.

- The Company continues to enroll the sixth cohort in the Phase 1/2 COMPOSE Study to further evaluate formulation refinement and pegtibatinase dosing.

PharmaKrysto Ltd Collaboration Agreement

- In March 2022, the Company entered into a collaboration agreement with PharmaKrysto Ltd, a privately held biopharmaceutical company, to advance discovery efforts around a series of novel compounds with the potential to target cystine stone prevention for patients living with cystinuria.

Conference Call Information

Travere Therapeutics will host a conference call and webcast today, Thursday, May 5, 2022 at 4:30 p.m. ET to discuss company updates as well as first quarter 2022 financial results. To participate in the conference call, dial +1 (800) 289-0720 (U.S.) or +1 (313) 209-5140 (International), confirmation code 7273768 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 5, 2022 to 7:30 p.m. ET, May 12, 2022. The replay number is +1 (888) 203-1112 (U.S.) or +1 (719) 457-0820 (International), confirmation code 7273768.

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 amortization and depreciation 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 "on-track", "positioned", "look forward to", "may", "might", "believes", "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 expectations around the FDA's timeline for providing notice of acceptance for filing of the NDA for sparsentan for IgAN and the outcome thereof; the Company's ability to execute towards its goal of making sparsentan a new treatment standard for rare kidney disorders, if approved; the Company's expectations for a potential commercial launch of sparsentan for IgAN, and the timing thereof; the ability to submit for accelerated approval in FSGS following regulatory engagement around additional eGFR data, as well as plans for MAA submissions in Europe for FSGS and IgAN; references to the efficacy, safety and tolerability profile of sparsentan based on the preliminary data from the DUPLEX and PROTECT Studies' interim analyses; the Company's expectations around timelines for topline results from the confirmatory endpoint analyses for the PROTECT and DUPLEX Studies; the potential establishment of a pivotal development program to support potential approval of pegtibatinase for the treatment of HCU; and the ability to advance discovery efforts around a series of novel compounds with the potential to target cystine stone prevention for patients living with cystinuria. 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; the 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 the 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 NDA submission of IgAN or that if it does, that it will grant such application priority review, that the Company will submit an application for accelerated approval for sparsentan in FSGS, 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, including under the heading "Risk Factors", 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, 2022	December 31, 2021
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 256,572	$ 165,753
Marketable debt securities, at fair value	346,818	387,129
Accounts receivable, net	14,675	15,914
Inventory, net	6,521	7,313
Prepaid expenses and other current assets	6,507	6,718
Total current assets	631,093	582,827
Property and equipment, net	10,602	11,106
Operating lease right of use assets	22,557	23,196
Intangible assets, net	153,321	148,435
Other assets	10,832	11,069
Total assets	$ 828,405	$ 776,633
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 18,434	$ 15,144
Accrued expenses	69,240	75,180
Deferred revenue, current portion	14,229	16,268
Business combination-related contingent consideration	7,500	7,400
Operating lease liabilities, current portion	4,014	3,908
Other current liabilities	6,056	6,188
Total current liabilities	119,473	124,088
Convertible debt	374,333	226,581
Deferred revenue, less current portion	19,669	20,379
Business combination-related contingent consideration, less current portion	66,000	59,700
Operating lease liabilities, less current portion	30,439	31,497
Other non-current liabilities	10,350	12,276
Total liabilities	620,264	474,521
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2022 and December 31, 2021	—	—
Common stock $0.0001 par value; 200,000,000 shares authorized; 63,510,277 and 62,491,498 issued and outstanding as of March 31, 2022 and December 31, 2021, respectively	6	6
Additional paid-in capital	1,021,542	1,068,634
Accumulated deficit	(811,712)	(765,966)
Accumulated other comprehensive loss	(1,695)	(562)
Total stockholders' equity	208,141	302,112
Total liabilities and stockholders' equity	$ 828,405	$ 776,633

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,	
	2022	2021
	(unaudited)	
Net product sales:		
Thiola/Thiola EC	$ 21,368	$ 25,443
Bile acid products	25,075	21,964
Total net product sales	46,443	47,407
License and collaboration revenue	2,044	—
Total revenue	48,487	47,407

Operating expenses:		
Cost of goods sold	2,138	1,645
Research and development	56,611	47,946
Selling, general and administrative	46,788	36,778
Change in fair value of contingent consideration	9,080	8,587
Total operating expenses	114,617	94,956
Operating loss	(66,130)	(47,549)
Other income (expenses), net:		
Interest income	278	409
Interest expense	(2,515)	(5,321)
Loss on early extinguishment of debt	(7,578)	—
Other income (expense), net	26	(1,093)
Total other expense, net	(9,789)	(6,005)
Loss before income tax provision	(75,919)	(53,554)
Income tax provision	(52)	(313)
Net loss	$ (75,971)	$ (53,867)
Per share data:		
Basic and diluted net loss per common share	$ (1.20)	$ (0.96)
Basic and diluted weighted average common shares outstanding	63,132,841	56,268,508

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,	
	2022	**2021**
GAAP operating loss	$ (66,130)	$ (47,549)
R&D operating expense	(56,611)	(47,946)
Stock compensation	3,168	3,002
Amortization & depreciation	286	286
Subtotal non-GAAP items	3,454	3,288
Non-GAAP R&D expense	(53,157)	(44,658)
SG&A operating expense	(46,788)	(36,778)
Stock compensation	5,018	4,692
Amortization & depreciation	6,806	5,789
Subtotal non-GAAP items	11,824	10,481
Non-GAAP SG&A expense	(34,964)	(26,297)
Change in fair value of contingent consideration	9,080	8,587
Subtotal non-GAAP items	24,358	22,356
Non-GAAP operating loss	**$ (41,772)**	**$ (25,193)**
GAAP net income (loss)	**$ (75,971)**	**$ (53,867)**
Non-GAAP operating loss adjustments	24,358	22,356

Income tax provision (benefit)	52	313
Non-GAAP net loss	**$ (51,561)**	**$ (31,198)**
Per share data:		
Basic and diluted net loss per common share	$ (0.82)	$ (0.55)
Basic and diluted weighted average common shares outstanding	63,132,841	56,268,508

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

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