Travere Therapeutics Reports Third Quarter 2022 Financial Results

October 27, 2022

EMA accepted for review the Conditional Marketing Authorization application for sparsentan for IgAN in Europe; review decision expected in second half of 2023

New PDUFA target action date for sparsentan in IgAN set for February 17, 2023

Net product sales of $50.8 million for the third quarter of 2022

SAN DIEGO, Oct. 27, 2022 (GLOBE NEWSWIRE) -- Travere Therapeutics, Inc. (NASDAQ: TVTX) today reported its third quarter 2022 financial results and provided a corporate update.

- European Medicines Agency (EMA) accepted for review the conditional marketing authorization (CMA) application for sparsentan for the treatment of IgAN in Europe; review decision on potential approval expected in second half of 2023
- U.S. Food and Drug Administration (FDA) confirmed three-month extension of New Drug Application (NDA) review process for sparsentan in IgA nephropathy (IgAN); new Prescription Drug User Fee Act (PDUFA) target action date of February 17, 2023, assigned
- Breakthrough Therapy Designation granted to pegtibatinase development program for classical homocystinuria (HCU)
- Total revenue for the third quarter 2022 was $53.5 million, consisting of $50.8 million in net product sales and $2.7 million in licensing and collaboration revenue
- Cash, cash equivalents and marketable securities, as of September 30, 2022, totaled $506.3 million

“During the third quarter we continued to execute towards our vision of being a leader in the global rare disease community,” said Eric Dube, Ph.D., president and chief executive officer of Travere Therapeutics. “Our IgA nephropathy and FSGS programs continue to advance with the goal of positioning sparsentan to become the first and only dual-acting, non-immunosuppressive treatment option for people living with rare kidney disorders. With the new PDUFA target action date confirmed in the U.S. and the acceptance of the CMA application in Europe in the third quarter, we are well positioned for potential approvals of sparsentan for the treatment of IgA nephropathy in both the U.S. and Europe next year. In the U.S., we have already built upon our strong commercial foundation to establish a field team with extensive nephrology experience. Additionally, we continue to look forward to the upcoming readout of our DUPLEX Study of sparsentan in FSGS in the first half of next year, as well as the potential to advance pegtibatinase into a pivotal trial for HCU in 2023.”

Quarter Ended September 30, 2022

Net product sales for the third quarter of 2022 were $50.8 million, compared to $54.2 million for the same period in 2021. For the nine months ended September 30, 2022, net product sales were $148.2 million, compared to $156.2 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 third quarter of 2022 were $59.3 million, compared to $48.4 million for the same period in 2021. For the nine months ended September 30, 2022, R&D expenses were $175.5 million, compared to $148.2 million for the same period in 2021. The difference is largely attributable to the continued advancement of the Company’s sparsentan and pegtibatinase programs, including increased headcount and medical affairs activities. On a non-GAAP adjusted basis, R&D expenses were $54.0 million for the third quarter of 2022, compared to $45.2 million for the same period in 2021.

Selling, general and administrative (SG&A) expenses for the third quarter of 2022 were $57.5 million, compared to $36.1 million for the same period in 2021. For the nine months ended September 30, 2022, SG&A expenses were $157.3 million, compared to $107.8 million for the same period in 2021. The difference is largely attributable to commercial launch preparations, including additional field-based headcount. On a non-GAAP adjusted basis, SG&A expenses were $45.4 million for the third quarter of 2022, compared to $25.5 million for the same period in 2021.

Total other expense, net, for the third quarter of 2022 was $1.4 million, compared to $3.9 million for the same period in 2021. The difference is largely attributable to increased interest income and lower interest expense during the period.

Net loss for the third quarter of 2022 was $69.7 million, or $1.09 per basic share, compared to a net loss of $35.6 million, or $0.59 per basic share for the same period in 2021. For the nine months ended September 30, 2022, net loss was $212.7 million, compared to $128.5 million for the same period in 2021. On a non-GAAP adjusted basis, net loss for the third quarter of 2022 was $48.9 million, or $0.76 per basic share, compared to a net loss of $7.9 million, or $0.13 per basic share for the same period in 2021.

As of September 30, 2022, the Company had cash, cash equivalents and marketable securities of $506.3 million.

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 May 2022, the FDA accepted and granted Priority Review of the Company's NDA under Subpart H for accelerated approval of sparsentan for the treatment of IgAN. The Company recently announced that as part of its late-cycle review interactions the FDA has requested that the Company update its proposed Risk Evaluation Mitigation Strategy (REMS) to include liver monitoring for sparsentan consistent with certain other approved products in the endothelin receptor antagonist class. The Company recently submitted an updated REMS and has received a new PDUFA target action date of February 17, 2023.

- In August 2022, the Company and its partner CSL Vifor announced that the EMA has accepted for review the CMA application for sparsentan for the treatment of IgAN. The EMA will review the application under the centralized marketing authorization procedure and a review decision on a potential approval is expected in the second half of 2023. Pending completion of the DUPLEX Study in focal segmental glomerulosclerosis (FSGS) and data supportive of approval, a subsequent variation of sparsentan for the treatment of FSGS is targeted for submission by the end of 2023.

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 showed 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. The Company anticipates having topline data from the DUPLEX Study, including full two-year eGFR data, in the first half of 2023 and being in position to submit a supplemental NDA for traditional approval in the second half of next year.

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 1.5 mg/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. As of the data cut-off, pegtibatinase has been generally well-tolerated. Enrollment activities continue for the sixth cohort in the Phase 1/2 COMPOSE Study to further evaluate formulation refinement and pegtibatinase dosing.

- In August 2022, the FDA granted Breakthrough Therapy Designation to the pegtibatinase program for the treatment of HCU. The Breakthrough Therapy Designation is supported by data from the ongoing Phase 1/2 COMPOSE Study of pegtibatinase in patients with HCU, as well as data from the Company's ongoing natural history study. To date, the pegtibatinase program has been granted Breakthrough Therapy, Rare Pediatric Disease and Fast Track designations by the FDA, as well as Orphan Drug designation in the U.S. and Europe.

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

Conference Call Information

Travere Therapeutics will host a conference call and webcast today, Thursday, October 27, 2022 at 4:30 p.m. ET to discuss company updates as well as third quarter 2022 financial results. To participate in the conference call, dial +1 (888) 394-8218 (U.S.) or +1 (323) 794-2551 (International), confirmation code 3327353 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 90 days.

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 likelihood of the FDA's potential approval of sparsentan for IgAN by the February 17, 2023 target action date or at all; EMA's potential approval of sparsentan for IgAN during the second half of 2023 or at all; the Company's goal of goal of positioning sparsentan to become the first and only dual-acting, non-immunosuppressive treatment option for people living with rare kidney disorders, if approved; the Company's expectations for a commercial launch of sparsentan for IgAN, if approved; expectations regarding the future conduct of the ongoing PROTECT and DUPLEX studies and timing for the topline eGFR endpoint analyses; the ability to submit for traditional approval in FSGS following the completion of the DUPLEX Study and expectations regarding the timing thereof, as well as plans for a potential regulatory submission of sparsentan in Europe for FSGS and the timing thereof; 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 ability to engage with regulators to establish next steps for the design of a pivotal development program to ultimately support potential approvals of pegtibatinase for the treatment of HCU; and the potential for pegtibatinase to become the first disease modifying therapy for people living with 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 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; 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 traditional 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 review process for the sparsentan IgAN NDA will remain on track for the FDA's extended target action date, that the FDA will grant accelerated approval of sparsentan for IgAN within the extended target action date, or at all, or that the DUPLEX Study will support an application for traditional review or that sparsentan will be approved for FSGS. There is also no guarantee that the results from the ongoing clinical study of pegtibatinase will be positive or that the Company will be able to align with regulators on the design of a pivotal program for pegtibatinase for HCU. 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.

Contact:

Investors:
Naomi Eichenbaum
Vice President, Investor Relations
888-969-7879
IR@travere.com

Media:
Nivi Nehra
Vice President, Corporate Communications
888-969-7879
mediarelations@travere.com

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

	September 30, 2022	December 31, 2021
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 151,337	$ 165,753
Marketable debt securities, at fair value	354,989	387,129
Accounts receivable, net	12,670	15,914
Inventory, net	7,297	7,313
Prepaid expenses and other current assets	8,549	6,718
Total current assets	534,842	582,827
Property and equipment, net	9,557	11,106
Operating lease right of use assets	21,253	23,196
Intangible assets, net	149,469	148,435
Other assets	11,094	11,069
Total assets	$ 726,215	$ 776,633
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 14,010	$ 15,144
Accrued expenses	88,383	75,180
Deferred revenue, current portion	11,383	16,268
Business combination-related contingent consideration, current portion	7,300	7,400
Operating lease liabilities, current portion	4,233	3,908
Other current liabilities	5,723	6,188
Total current liabilities	131,032	124,088
Convertible debt	375,117	226,581
Deferred revenue, less current portion	12,919	20,379
Business combination-related contingent consideration, less current portion	68,900	59,700
Operating lease liabilities, less current portion	28,267	31,497
Other non-current liabilities	9,304	12,276
Total liabilities	625,539	474,521
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of September 30, 2022 and December 31, 2021	—	—
Common stock $0.0001 par value; 200,000,000 shares authorized; 64,150,510, and 62,491,498 issued and outstanding as of September 30, 2022 and December 31, 2021, respectively	6	6
Additional paid-in capital	1,048,767	1,068,634
Accumulated deficit	(948,400)	(765,966)
Accumulated other comprehensive loss	303	(562)
Total stockholders' equity	100,676	302,112
Total liabilities and stockholders' equity	$ 726,215	$ 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 September 30,		Nine Months Ended September 30,	
	2022	**2021**	**2022**	**2021**
	(unaudited)			
Net product sales:				
Bile acid products	$ 25,420	$ 24,353	$ 76,029	$ 71,291
Tiopronin products	25,370	29,821	72,154	84,907
Total net product sales	50,790	54,174	148,183	156,198
License and collaboration revenue	2,706	14,043	7,967	14,043
Total revenue	53,496	68,217	156,150	170,241
Operating expenses:				
Cost of goods sold	1,675	1,592	5,864	4,888
Research and development	59,256	48,407	175,548	148,160
Selling, general and administrative	57,519	36,065	157,286	107,808
Change in fair value of contingent consideration	3,180	13,864	17,167	23,960
Total operating expenses	121,630	99,928	355,865	284,816
Operating loss	(68,134)	(31,711)	(199,715)	(114,575)
Other income (expenses), net:				
Interest income	2,101	360	3,161	1,757
Interest expense	(2,892)	(4,899)	(8,379)	(15,072)
Loss on early extinguishment of debt	—	—	(7,578)	—
Other income (expense), net	(586)	654	102	(223)
Total other expense, net	(1,377)	(3,885)	(12,694)	(13,538)
Loss before income tax provision	(69,511)	(35,596)	(212,409)	(128,113)
Income tax provision	(145)	(43)	(250)	(405)
Net loss	$ (69,656)	$ (35,639)	$ (212,659)	$ (128,518)
Per share data:				
Basic and diluted net loss per common share	$ (1.09)	$ (0.59)	$ (3.34)	$ (2.17)
Basic and diluted weighted average common shares outstanding	64,033,759	60,803,045	63,604,962	59,230,881

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,	
	2022	**2021**	**2022**	**2021**
GAAP operating loss	$ (68,134)	$ (31,711)	$ (199,715)	$ (114,575)
R&D operating expense	(59,256)	(48,407)	(175,548)	(148,160)
Stock compensation	3,393	2,630	10,245	8,477
Amortization & depreciation	1,906	614	3,817	1,188
Subtotal non-GAAP items	5,299	3,244	14,062	9,665
Non-GAAP R&D expense	(53,957)	(45,163)	(161,486)	(138,495)
SG&A operating expense	(57,519)	(36,065)	(157,286)	(107,808)

Stock compensation	5,433	4,356	19,404	13,713
Amortization & depreciation	6,672	6,250	19,961	18,369
Subtotal non-GAAP items	12,105	10,606	39,365	32,082
Non-GAAP SG&A expense	(45,414)	(25,459)	(117,921)	(75,726)
Change in fair value of contingent consideration	3,180	13,864	17,167	23,960
Subtotal non-GAAP items	20,584	27,714	70,594	65,707
Non-GAAP operating loss	**$ (47,550)**	**$ (3,997)**	**$ (129,121)**	**$ (48,868)**
GAAP net loss	**$ (69,656)**	**$ (35,639)**	**$ (212,659)**	**$ (128,518)**
Non-GAAP operating loss adjustments	20,584	27,714	70,594	65,707
Income tax provision	145	43	250	405
Non-GAAP net loss	**$ (48,927)**	**$ (7,882)**	**$ (141,815)**	**$ (62,406)**
Per share data:				
Basic and diluted net loss per common share	$ (0.76)	$ (0.13)	$ (2.23)	$ (1.05)
Basic and diluted weighted average common shares outstanding	64,033,759	60,803,045	63,604,962	59,230,881

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

Source: Travere Therapeutics, Inc.