



## Traverse Therapeutics Reports Fourth Quarter and Full Year 2020 Financial Results

March 1, 2021

*Recently reported that ongoing DUPLEX Study of sparsentan in FSGS achieved interim proteinuria endpoint*

*Preparations underway for meetings with regulatory agencies in first half of 2021 to discuss planned accelerated approval submissions for FSGS*

*Pivotal PROTECT Study of sparsentan in IgA nephropathy on track to report topline proteinuria results in the third quarter of 2021*

*Net product sales increased 13 percent to \$198 million for the full year 2020*

*February common stock offering generated net proceeds of \$189 million*

SAN DIEGO, March 01, 2021 (GLOBE NEWSWIRE) -- Traverse Therapeutics, Inc. (NASDAQ: TVTX) today reported its fourth quarter and full year 2020 financial results and provided a corporate update.

- The Company recently reported 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) after 36 weeks of treatment
- The Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN) remains on-track to report topline data from the 36-week proteinuria analysis in the third quarter of 2021
- Completed acquisition of TVT-058 (previously OT-58), an investigational human enzyme replacement therapy with disease modifying potential in Phase 1/2 development for the treatment of classical homocystinuria (HCU)
- Net product sales for the fourth quarter of 2020 were \$51.0 million, compared to \$46.7 million for the same period in 2019
- Net product sales for the full year 2020 were \$198.3 million, compared to \$175.3 million for the full year 2019
- Cash, cash equivalents and marketable securities, as of December 31, 2020, totaled \$361.6 million; an additional approximately \$189 million in net proceeds received in February 2021 from common stock offering

"In 2020, our organization executed on all of our key objectives despite the challenges of the ongoing pandemic. This resulted in achieving enrollment milestones in our pivotal studies of sparsentan in FSGS and IgAN at or ahead of schedule, diversifying our pipeline with the addition of TVT-058 and serving more patients than ever before with our approved products," said Eric Dube, Ph.D., chief executive officer of Traverse Therapeutics. "Led by the interim readouts from our Phase 3 studies of sparsentan, we expect 2021 to be a pivotal year for Traverse. We were very pleased to begin the year with the DUPLEX Study in FSGS achieving its pre-specified interim proteinuria endpoint. Reaching this clinical milestone positions us to engage with regulators in pursuit of submissions for accelerated approval as we continue efforts to prepare our organization for the future commercialization of sparsentan, if approved. We are also looking forward to the interim proteinuria assessment from the PROTECT Study in IgAN during the third quarter, which could potentially position us to ultimately deliver sparsentan as a new treatment standard in both FSGS and IgAN."

### Fourth Quarter and Full Year 2020 Financial Results

Net product sales for the fourth quarter of 2020 were \$51.0 million, compared to \$46.7 million for the same period in 2019. For the full year 2020, net product sales were \$198.3 million, compared to \$175.3 million for the same period in 2019. The increase in net product sales is attributable to organic growth across the Company's commercial products. In 2021, the Company anticipates mid-single-digit percentage growth in net product sales compared to 2020.

Research and development (R&D) expenses for the fourth quarter of 2020 were \$38.4 million, compared to \$36.4 million for the same period in 2019. For the full year 2020, R&D expenses were \$131.8 million, compared to \$141.0 million for the same period in 2019. The difference is largely attributable to the discontinuation of the fosmetpantotenate development program during the fourth quarter of 2019. On a non-GAAP adjusted basis, R&D expenses were \$35.7 million for the fourth quarter of 2020, compared to \$34.5 million for the same period in 2019.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2020 were \$35.7 million, compared to \$27.5 million for the same period in 2019. For the full year 2020, SG&A expenses were \$135.8 million, compared to \$129.0 million for the same period in 2019. The difference is largely attributable to increased headcount as a result of the Company's operational growth, and professional fees. On a non-GAAP adjusted basis, SG&A expenses were \$25.5 million for the fourth quarter of 2020, compared to \$19.6 million for the same period in 2019.

The Company incurred a \$97.1 million IPR&D expense during the fourth quarter of 2020 as a result of its acquisition of the TVT-058 development program in November of 2020.

Total other expense, net, for the fourth quarter of 2020 was \$3.5 million, compared to \$2.1 million for the same period in 2019. The difference is largely attributable to a reduction in interest income.

Net loss for the fourth quarter of 2020 was \$121.6 million, or \$2.37 per basic share, compared to a net loss of \$30.3 million, or \$0.70 per basic share for the same period in 2019. For the full year 2020, net loss was \$169.4 million, compared to \$146.4 million for the same period in 2019. On a non-GAAP adjusted basis, net loss for the fourth quarter of 2020 was \$112.9 million, or \$2.20 per basic share, compared to a net loss of \$11.2 million, or \$0.26 per basic share for the same period in 2019.

As of December 31, 2020, the Company had cash, cash equivalents and marketable securities of \$361.6 million. In February 2021, the Company completed a common stock offering with net proceeds of approximately \$189 million.

## 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)  $\leq 1.5$  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 will continue its engagement with regulators in the first half of 2021 to discuss the ongoing study and 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, with the available data set. 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 remains on track to complete enrollment in 2021. 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.
- In the first quarter of 2021, the U.S. Food and Drug Administration (FDA) and the European Commission granted orphan designation to sparsentan for the treatment of IgAN.

### *TVT-058*

- In November 2020, the Company completed its acquisition of Orphan Technologies Limited and its TVT-058 clinical development program. TVT-058 is a novel investigational human enzyme replacement therapy advancing in clinical development for the treatment of classical HCU. The Company anticipates preliminary data from the ongoing Phase 1/2 study in 2021 and is monitoring the potential impact of the evolving COVID-19 pandemic on this timing. If ultimately approved, TVT-058 has the potential to become the first disease modifying therapy for people living with HCU.

## Conference Call Information

Travere Therapeutics will host a conference call and webcast today, Monday, March 1, 2021 at 4:30 p.m. ET to discuss company updates as well as fourth quarter and full year 2020 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 3397951 shortly before 4:30 p.m. ET. The webcast can be accessed at [travere.com](http://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, March 1, 2021 to 7:30 p.m. ET, March 8, 2021. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 3397951.

## 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 Traver Therapeutics

At Traver 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](http://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 continuing its engagement with regulators; expectations regarding anticipated accelerated approval regulatory submissions for sparsentan in FSGS based on the available data set from the DUPLEX interim analysis; the potential for the Company to ultimately deliver sparsentan as a new treatment standard in both FSGS and IgAN; 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, based on the existing data, 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. AND SUBSIDIARIES**  
**CONSOLIDATED BALANCE SHEETS**  
*(in thousands, except share amounts)*

	December 31, 2020	December 31, 2019
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 84,772	\$ 62,436
Available-for-sale debt securities, at fair value (amortized cost \$276,111, allowance for credit losses of \$0 as of December 31, 2020; amortized cost \$335,206, allowance for credit losses of \$0 as of December 31, 2019)	276,817	336,088
Accounts receivable, net	15,925	18,048

Inventory, net	7,608	6,082
Prepaid expenses and other current assets	8,143	5,015
Tax receivable	17,142	1,395
<b>Total current assets</b>	<b>410,407</b>	<b>429,064</b>
Property and equipment, net	9,418	2,891
Other assets	33,489	14,709
Intangible assets, net	153,189	157,200
Goodwill	936	936
<b>Total assets</b>	<b>\$ 607,439</b>	<b>\$ 604,800</b>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 12,133	\$ 26,614
Accrued expenses	56,793	51,745
Other current liabilities	6,334	8,590
Business combination-related contingent consideration	17,400	8,500
<b>Total current liabilities</b>	<b>92,660</b>	<b>95,449</b>
Convertible debt	215,339	204,861
Other non-current liabilities	40,527	20,894
Business combination-related contingent consideration, less current portion	47,700	62,400
<b>Total liabilities</b>	<b>396,226</b>	<b>383,604</b>
<b>Stockholders' Equity:</b>		
Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2020 and 2019, respectively	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 52,248,431 and 43,088,921 issued and outstanding as of December 31, 2020 and 2019, respectively	5	4
Additional paid-in capital	797,985	636,910
Accumulated deficit	(585,875)	(416,444)
Accumulated other comprehensive (loss) income	(902)	726
Total stockholders' equity	211,213	221,196
<b>Total liabilities and stockholders' equity</b>	<b>\$ 607,439</b>	<b>\$ 604,800</b>

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

**TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES**  
**CONSOLIDATED STATEMENT OF OPERATIONS**  
*(in thousands, except share and per share data)*

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
	<i>(unaudited)</i>			
Net product sales:				
Thiola/Thiola EC	\$ 28,311	\$ 26,246	\$ 108,883	\$ 95,638
Bile acid products	22,672	20,442	89,438	79,700
Total net product sales	50,983	46,688	198,321	175,338
Operating expenses:				
Cost of goods sold	2,073	1,725	6,126	5,234
Research and development	38,385	36,366	131,773	140,963
Selling, general and administrative	35,738	27,533	135,799	128,951
Change in fair value of contingent consideration	(3,794)	9,231	3,655	15,051
Impairment of L-UDCA IPR&D intangible asset	—	—	—	25,500
Write off of L-UDCA contingent consideration	—	—	—	(18,000)
Acquired IPR&D expense	97,131	—	97,131	—
Impairment of long-term investment	—	—	—	15,000
Total operating expenses	169,533	74,855	374,484	312,699
Operating loss	(118,550)	(28,167)	(176,163)	(137,361)

Other Income (expense), net:				
Other income (expense), net	631	359	1,420	(314)
Interest income	588	2,180	5,003	10,055
Interest expense	(4,762)	(4,599)	(19,050)	(18,828)
Total other expense, net	(3,543)	(2,060)	(12,627)	(9,087)
Loss before benefit (provision) for income taxes	(122,093)	(30,227)	(188,790)	(146,448)
Income tax benefit (provision)	471	(32)	19,359	21
Net loss	<u>\$ (121,622)</u>	<u>\$ (30,259)</u>	<u>\$ (169,431)</u>	<u>\$ (146,427)</u>
Net earnings (loss) per common share, basic	<u>\$ (2.37)</u>	<u>\$ (0.70)</u>	<u>\$ (3.56)</u>	<u>\$ (3.46)</u>
Net earnings (loss) per common share, diluted	<u>\$ (2.37)</u>	<u>\$ (0.70)</u>	<u>\$ (3.56)</u>	<u>\$ (3.46)</u>
Weighted average common shares outstanding, basic	<u>51,264,029</u>	<u>43,023,479</u>	<u>47,539,631</u>	<u>42,339,961</u>
Weighted average common shares outstanding, diluted	51,264,029	43,023,479	47,539,631	42,339,961

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

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
<b>GAAP operating loss</b>	<b>\$ (118,550)</b>	<b>\$ (28,167)</b>	<b>\$ (176,163)</b>	<b>\$ (137,361)</b>
R&D operating expense	(38,385)	(36,366)	(131,773)	(140,963)
Stock compensation	2,399	1,609	9,367	6,910
Amortization & depreciation	293	292	1,163	1,159
Subtotal non-GAAP items	<u>2,692</u>	<u>1,901</u>	<u>10,530</u>	<u>8,069</u>
Non-GAAP R&D expense	<u>(35,693)</u>	<u>(34,465)</u>	<u>(121,243)</u>	<u>(132,894)</u>
SG&A operating expense	(35,738)	(27,533)	(135,799)	(128,951)
Stock compensation	3,953	2,888	14,247	14,195
Amortization & depreciation	6,295	4,998	23,371	19,249
Subtotal non-GAAP items	<u>10,248</u>	<u>7,886</u>	<u>37,618</u>	<u>33,444</u>
Non-GAAP SG&A expense	<u>(25,490)</u>	<u>(19,647)</u>	<u>(98,181)</u>	<u>(95,507)</u>
Change in fair value of contingent consideration	(3,794)	9,231	3,655	15,051
Subtotal non-GAAP items	<u>9,146</u>	<u>19,018</u>	<u>51,803</u>	<u>56,564</u>
<b>Non-GAAP operating loss</b>	<b>\$ (109,404)</b>	<b>\$ (9,149)</b>	<b>\$ (124,360)</b>	<b>\$ (80,797)</b>
<b>GAAP net loss</b>	<b>\$ (121,622)</b>	<b>\$ (30,259)</b>	<b>\$ (169,431)</b>	<b>\$ (146,427)</b>
Non-GAAP operating loss adjustments	9,146	19,018	51,803	56,564
Income tax provision	(471)	32	(19,359)	(21)
<b>Non-GAAP net loss</b>	<b>\$ (112,947)</b>	<b>\$ (11,209)</b>	<b>\$ (136,987)</b>	<b>\$ (89,884)</b>
<b>Per share data:</b>				
Net earnings per common share, basic	<u>\$ (2.20)</u>	<u>\$ (0.26)</u>	<u>\$ (2.88)</u>	<u>\$ (2.12)</u>
Weighted average common shares outstanding, basic	<u>51,264,029</u>	<u>43,023,479</u>	<u>47,539,631</u>	<u>42,339,961</u>

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

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Source: Travere Therapeutics, Inc.