



Traverse Therapeutics Reports Fourth Quarter and Full Year 2021 Financial Results

February 24, 2022

NDA submission of sparsentan in IgA nephropathy on-track for first quarter 2022

Company positioned for potential NDA submission for accelerated approval of sparsentan in FSGS and combined IgA nephropathy and FSGS MAA submission mid-year 2022

Recently reported positive topline results from the ongoing Phase 1/2 COMPOSE Study of pegtibatinase in classical homocystinuria

Net product sales increased six percent to \$211 million for the full year 2021

SAN DIEGO, Feb. 24, 2022 (GLOBE NEWSWIRE) -- Traverse Therapeutics, Inc. (NASDAQ: TVTX) today reported its fourth quarter and full year 2021 financial results and provided a corporate update.

- The Company is on-track to submit a New Drug Application (NDA) for accelerated approval of sparsentan in IgA nephropathy (IgAN) in the first quarter of 2022
- Preparations underway to submit NDA for accelerated approval of sparsentan for focal segmental glomerulosclerosis (FSGS) and combined IgAN and FSGS Marketing Authorisation Application (MAA), pending supportive estimated glomerular filtration (eGFR) data from ongoing Phase 3 DUPLEX Study
- Company is engaging with regulators to establish next steps for a pivotal development program for pegtibatinase following recently reported positive topline results from the ongoing Phase 1/2 COMPOSE Study
- Net product sales for the fourth quarter 2021 were \$54.6 million, compared to \$51.0 million for the same period in 2020
- Total revenue for the full year 2021 was \$227.5 million, consisting of \$210.8 million in net product sales and \$16.7 million in licensing and collaboration revenue
- Cash, cash equivalents and marketable securities, as of December 31, 2021, totaled \$552.9 million

"2021 was an outstanding year for Traverse. Our pipeline of potential first in class rare disease treatments delivered three positive topline readouts from our ongoing studies and we established regulatory pathways to potential accelerated approvals in both IgA nephropathy and FSGS for sparsentan. We also continued to execute with our commercial products, achieving six percent organic growth in net product sales for the year," said Eric Dube, Ph.D., chief executive officer of Traverse Therapeutics. "We are carrying this positive momentum into 2022. We remain on-track for our planned regulatory submissions, including the NDA for sparsentan in IgA nephropathy in the first quarter. We also look forward to advancing our pegtibatinase program towards pivotal development and building upon our commercial expertise in rare nephrology to ready for a potential first launch of sparsentan, if approved, as early as the end of this year."

Fourth Quarter and Full Year 2021 Financial Results

Net product sales for the fourth quarter of 2021 were \$54.6 million, compared to \$51.0 million for the same period in 2020. For the full year 2021, net product sales were \$210.8 million, compared to \$198.3 million for the same period in 2020. The increase in net product sales was attributable to growth across the Company's commercial products.

Research and development (R&D) expenses for the fourth quarter of 2021 were \$62.2 million, compared to \$38.4 million for the same period in 2020. For the full year 2021, R&D expenses were \$210.3 million, compared to \$131.8 million for the same period in 2020. 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 \$57.7 million for the fourth quarter of 2021, compared to \$35.7 million for the same period in 2020.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2021 were \$42.1 million, compared to \$35.7 million for the same period in 2020. For the full year 2021, SG&A expenses were \$149.9 million, compared to \$135.8 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 \$30.9 million for the fourth quarter of 2021, compared to \$25.5 million for the same period in 2020.

Total operating expenses for the fourth quarter of 2021 were \$104.4 million, compared to \$169.5 million for the same period in 2020. For the full year 2021, total operating expenses were \$389.3 million, compared to \$374.5 million for the same period in 2020. The difference in the fourth quarter of 2021 is largely attributable to acquired IPR&D expense that was recognized as a result of the Company's acquisition of the pegtibatinase development program in the same period of 2020.

Total other expense, net, for the fourth quarter of 2021 was \$4.4 million, compared to \$3.5 million for the same period in 2020. The difference is largely attributable to a reduction in interest income.

Net loss for the fourth quarter of 2021 was \$51.6 million, or \$0.84 per basic share, compared to a net loss of \$121.6 million, or \$2.37 per basic share for the same period in 2020. For the full year 2021, net loss was \$180.1 million, compared to \$169.4 million for the same period in 2020. On a non-GAAP adjusted basis, net loss for the fourth quarter of 2021 was \$37.6 million, or \$0.61 per basic share, compared to a net loss of \$112.9 million, or \$2.20 per basic share for the same period in 2020.

As of December 31, 2021, the Company had cash, cash equivalents and marketable securities of \$552.9 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.
- The Company remains on-track to submit an NDA for accelerated approval of sparsentan for IgAN in the U.S. in the first quarter of 2022.
- In collaboration with its partner Vifor Pharma, the Company expects to submit a combined IgAN and FSGS MAA in mid-2022 for conditional marketing authorization of sparsentan in Europe.

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 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 of sparsentan in FSGS to the U.S. Food and Drug Administration (FDA) in the first half of 2022. Should additional eGFR data from the study be supportive as expected, the Company anticipates submitting an NDA for accelerated approval of sparsentan for FSGS in the U.S. in mid-2022.

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 classical homocystinuria (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% 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. Additional detailed study results from the first five dose cohorts in the ongoing Phase 1/2 COMPOSE Study are expected to be presented at a medical meeting in the first half of 2022.
- 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 has initiated enrollment for the sixth cohort in the Phase 1/2 COMPOSE Study to further evaluate formulation refinement and pegtibatinase dosing.

Conference Call Information

Traverse Therapeutics will host a conference call and webcast today, Thursday, February 24, 2022 at 4:30 p.m. ET to discuss company updates as well as fourth quarter and full year 2021 financial results. To participate in the conference call, dial +1 (855) 219-9219 (U.S.) or +1 (315) 625-6891 (International), confirmation code 6217409 shortly before 4:30 p.m. ET. The webcast can be accessed at traverse.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, February 24, 2022 to 7:30 p.m. ET, March 3, 2022. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 6217409.

Use of Non-GAAP Financial Measures

To supplement Traver'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. Traver's management regularly uses these supplemental non-GAAP financial measures internally to understand, manage and evaluate its business and make operating decisions. In addition, Traver 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 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 traver.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: expected sparsentan regulatory submissions in 2022, including the timing for the planned IgAN accelerated approval submission, the ability to submit for accelerated approval in FSGS, pending additional supportive eGFR data, as well as expectations regarding submitting a joint marketing authorization application in Europe for both FSGS and IgAN; the Company's expectations for a potential first launch of sparsentan, if approved, 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 and expectations that additional eGFR data from the DUPLEX Study will support an accelerated approval submission; the Company's expectations around timelines for topline results from the confirmatory endpoint analyses for the PROTECT and DUPLEX Studies; the Company's expectations around timelines for additional detailed study results from the ongoing Phase 1/2 study of pegtibatase in HCU; the potential establishment of a pivotal development program to support potential approval of pegtibatase for the treatment of HCU; and references to the potential for pegtibatase, if approved, to become the first disease modifying therapy for people living with classical HCU. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties associated with the regulatory review and approval process, including the Subpart H accelerated approval pathway in the United States and the conditional marketing authorization (CMA) pathway in the 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 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 pegtibatase 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. AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS
(in thousands, except share amounts)

	December 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	\$ 165,753	\$ 84,772
Marketable debt securities, at fair value	387,129	276,817
Accounts receivable, net	15,914	15,925
Inventory, net	7,313	7,608
Tax receivable	247	17,142
Prepaid expenses and other current assets	6,471	8,143
Total current assets	582,827	410,407
Property and equipment, net	11,106	9,418
Operating lease right of use assets	23,196	25,675
Intangible assets, net	148,435	154,125
Other assets	11,069	7,814
Total assets	\$ 776,633	\$ 607,439
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 15,144	\$ 12,133
Accrued expenses	75,180	56,793
Deferred revenue, current portion	16,268	—
Business combination-related contingent consideration	7,400	17,400
Operating lease liabilities, current portion	3,908	357
Other current liabilities	6,188	5,977
Total current liabilities	124,088	92,660
Convertible debt	226,581	215,339
Deferred revenue, less current portion	20,379	—
Business combination-related contingent consideration, less current portion	59,700	47,700
Operating lease liabilities, less current portion	31,497	28,336
Other non-current liabilities	12,276	12,191
Total liabilities	474,521	396,226
Stockholders' Equity:		
Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2021 and 2020	—	—
Common stock \$0.0001 par value; 200,000,000 and 100,000,000 shares authorized; 62,491,498 and 52,248,431 issued and outstanding as of December 31, 2021 and 2020, respectively	6	5
Additional paid-in capital	1,068,634	797,985
Accumulated deficit	(765,966)	(585,875)
Accumulated other comprehensive loss	(562)	(902)
Total stockholders' equity	302,112	211,213
Total liabilities and stockholders' equity	\$ 776,633	\$ 607,439

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,
	2021	2020
	2021	2020

(unaudited)

Net product sales:				
Tiopronin products	\$ 30,215	\$ 28,311	\$ 115,122	\$ 108,883
Bile acid products	24,363	22,672	95,654	89,438
Total net product sales	54,578	50,983	210,776	198,321
License and collaboration revenue	2,671	—	16,714	—
Total revenue	57,249	50,983	227,490	198,321
Operating expenses:				
Cost of goods sold	1,896	2,073	6,784	6,126
Research and development	62,168	38,385	210,328	131,773
Selling, general and administrative	42,075	35,738	149,883	135,799
Change in fair value of contingent consideration	(1,700)	(3,794)	22,260	3,655
Acquired IPR&D expense	—	97,131	—	97,131
Total operating expenses	104,439	169,533	389,255	374,484
Operating loss	(47,190)	(118,550)	(161,765)	(176,163)
Other Income (expense), net:				
Interest income	236	588	1,993	5,003
Interest expense	(5,069)	(4,762)	(20,141)	(19,050)
Other income (expense), net	454	631	231	1,420
Total other expense, net	(4,379)	(3,543)	(17,917)	(12,627)
Loss before (provision) benefit for income taxes	(51,569)	(122,093)	(179,682)	(188,790)
Income tax benefit (provision)	(4)	471	(409)	19,359
Net loss	<u>\$ (51,573)</u>	<u>\$ (121,622)</u>	<u>\$ (180,091)</u>	<u>\$ (169,431)</u>
Net earnings (loss) per common share, basic	<u>\$ (0.84)</u>	<u>\$ (2.37)</u>	<u>\$ (3.01)</u>	<u>\$ (3.56)</u>
Net earnings (loss) per common share, diluted	<u>\$ (0.84)</u>	<u>\$ (2.37)</u>	<u>\$ (3.01)</u>	<u>\$ (3.56)</u>
Weighted average common shares outstanding, basic	<u>61,616,896</u>	<u>51,264,029</u>	<u>59,832,287</u>	<u>47,539,631</u>
Weighted average common shares outstanding, diluted	61,616,896	51,264,029	59,832,287	47,539,631

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,	
	2021	2020	2021	2020
GAAP operating loss	\$ (47,190)	\$ (118,550)	\$ (161,765)	\$ (176,163)
R&D operating expense	(62,168)	(38,385)	(210,328)	(131,773)
Stock compensation	4,155	2,399	12,632	9,367
Amortization & depreciation	293	293	1,481	1,163
Subtotal non-GAAP items	4,448	2,692	14,113	10,530
Non-GAAP R&D expense	(57,720)	(35,693)	(196,215)	(121,243)
SG&A operating expense	(42,075)	(35,738)	(149,883)	(135,799)
Stock compensation	4,421	3,953	18,134	14,247
Amortization & depreciation	6,768	6,295	25,137	23,371
Subtotal non-GAAP items	11,189	10,248	43,271	37,618
Non-GAAP SG&A expense	(30,886)	(25,490)	(106,612)	(98,181)
Change in fair value of contingent consideration	(1,700)	(3,794)	22,260	3,655

Subtotal non-GAAP items	13,937	9,146	79,644	51,803
Non-GAAP operating loss	\$ (33,253)	\$ (109,404)	\$ (82,121)	\$ (124,360)
GAAP net loss	\$ (51,573)	\$ (121,622)	\$ (180,091)	\$ (169,431)
Non-GAAP operating loss adjustments	13,937	9,146	79,644	51,803
Income tax provision	4	(471)	409	(19,359)
Non-GAAP net loss	\$ (37,632)	\$ (112,947)	\$ (100,038)	\$ (136,987)
Per share data:				
Basic and diluted net loss per common share	\$ (0.61)	\$ (2.20)	\$ (1.67)	\$ (2.88)
Basic and diluted weighted average common shares outstanding	61,616,896	51,264,029	59,832,287	47,539,631

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

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Source: Traverse Therapeutics, Inc.