



Voyager Therapeutics Reports Fourth Quarter and Full Year 2021 Financial and Operating Results

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License option agreement with Novartis for target-specific access to TRACER™ next-generation AAV capsids for potential use with three CNS targets plus options to access capsids for two additional targets

Novartis agreement marks second recent major transaction to leverage Voyager's TRACER technology following October 2021 Pfizer agreement

Strengthened leadership with appointment of Al Sandrock, Jr., M.D., Ph.D., to Board of Directors and Board Executive Committee, promotion of Robin Swartz to chief operating officer

Cash runway extended into 2024 with \$84 million aggregate upfront payments from Novartis and Pfizer transactions

Conference call and webcast today at 8:30 a.m. ET

CAMBRIDGE, Mass., March 08, 2022 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a gene therapy company developing life-changing treatments and next-generation adeno-associated virus (AAV) capsids, today reported fourth quarter and full year 2021 financial and operating results.

"We're extremely pleased with the value created by our TRACER™ capsid discovery platform, evidenced by the two major license option agreements with Novartis and Pfizer for target-specific access to our highly differentiated capsids, which affords us the opportunity to expand the impact of our technology through external program development by our partners. The potential of our novel TRACER capsids to improve transduction in target tissues and to minimize off target toxicities associated with conventional AAV delivery may enable a new wave of gene therapies to treat serious diseases," said Michael Higgins, interim CEO of Voyager. "We've also made important additions to our leadership team with Al Sandrock joining our Board and its Executive Committee, and the promotion of Robin Swartz to chief operating officer. We continue to leverage our proprietary AAV capsids to progress our pipeline and look forward to sharing new preclinical data from select programs and our TRACER platform at a medical conference this spring."

Target-Specific License Option Agreement with Novartis Expands Applications of TRACER Capsids

- Voyager today announced [a license option agreement](#) with Novartis AG (NYSE: NVS) through which Novartis may exercise options to license novel AAV capsids generated from Voyager's RNA-driven TRACER (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) capsid discovery platform for potential use with three undisclosed CNS targets and options to access capsids for two additional targets to be agreed on in the future, subject to availability. These targets are distinct from those being explored in Voyager's internal pipeline and other licensing agreements. Voyager retains global rights to its TRACER discovery platform and all capsids arising from it, subject to certain obligations under its agreement with Pfizer, as detailed below.
- Voyager will receive \$54 million up front and is entitled to receive up to \$37.5 million in exercise fees for three initial CNS targets, exercisable by Novartis within 12 months of signing. In addition, Novartis may elect to evaluate capsids for up to two additional targets, to be agreed upon in the future, subject to availability, for \$18 million upon selection of each target, and a \$12.5 million exercise fee for selection of a capsid for each target. Voyager is also eligible to earn up to \$1.5 billion in total development, regulatory, and commercial milestones for products utilizing Voyager-licensed capsids, as well as mid- to high-single-digit tiered royalties based on net sales of Novartis products incorporating the licensed capsids.

Transgene-Specific License Option Agreement with Pfizer Supports CNS and Cardiac Applications for TRACER

- In October, Voyager and Pfizer Inc. (NYSE: PFE) [announced](#) a license option agreement through which Pfizer may exercise options to license AAV capsids generated from the TRACER platform. Under the terms of the agreement, Pfizer received target-specific access to Voyager's TRACER capsids for one CNS and one cardiovascular target and the right to exercise options to license capsids for exclusive use in Pfizer's development of AAV gene therapies for each target. Voyager retains global rights to all TRACER capsids for use with other targets and to all other applications of its TRACER technology.
- Voyager received \$30 million upfront and is entitled to receive up to \$20 million in exercise fees in aggregate for two options exercisable by Pfizer within 12 months of signing, and is eligible to earn up to \$580 million in total development, regulatory, and commercial milestones, as well as mid- to high-single-digit tiered royalties based on net sales of Pfizer products incorporating the licensed capsids.

Additional Data from Evolving TRACER AAV9 and AAV5 Capsid Portfolio Planned for Spring Medical Conference

- Voyager continues to perform screening campaigns with its TRACER capsid discovery platform to identify additional proprietary AAV9- and AAV5-derived capsids and to refine capsids already identified to target multiple tissue and cell types

for use in gene therapies to treat a broad range of diseases. Numerous promising capsids have been identified and developed from these screens, which are undergoing testing across multiple non-human primate (NHP) and rodent species to evaluate their clinical translatability.

- Voyager previously [presented](#) data on a set of AAV9-derived TRACER capsids from its first campaign that demonstrated robust delivery across the blood-brain barrier and widespread CNS transduction in NHPs, compared to conventional AAV9 delivery, when dosed intravenously. In addition, another AAV9-derived TRACER capsid displayed strong cardiac transduction and significant dorsal root ganglia detargeting in NHPs, which may help avoid dose-related toxicities associated with AAV9 delivery.
- Voyager has identified a new array of TRACER AAV9 and AAV5 variants that have demonstrated improved CNS targeting in both NHPs and rodents with certain AAV TRACER capsids showing a preferential tropism for glial cells, which may enable more effective targeting of relevant cell types for certain CNS diseases. AAV5 capsids have been shown to have a low prevalence of preexisting neutralizing antibodies in the general population and significant manufacturing advantages over conventional AAV9. In addition, some of these variants have demonstrated cross-species translatability across primates and rodents.
- Voyager plans to present preclinical data on the performance of these TRACER capsids in NHP and mouse models at a spring medical conference.

Positive Preclinical Data from Vectorized Anti-HER2 Antibody Program Highlights Advancement of Rich Early-Stage Pipeline

- Voyager continues to advance its preclinical programs powered by its TRACER capsids. The Company believes these capsids may enable new, best-in-class gene therapy programs with systemic IV delivery with lower risk of dose-limiting toxicities. Our pipeline includes gene replacement programs for spinal muscular atrophy, diseases linked to GBA1 mutations (including Parkinson's disease, Lewy body dementia, and Gaucher disease), gene knockdown or silencing programs for SOD1 ALS and Huntington's disease, and vectorized antibody programs for HER2+ brain metastases and diseases associated with pathological tau (including Alzheimer's disease, frontal-temporal dementia, and progressive supranuclear palsy). Voyager plans to present further preclinical data from select programs at a spring medical conference.
- In November, the Company presented positive [preclinical data](#) demonstrating that IV dosing of a novel TRACER AAV vector encoding an anti-HER2 antibody payload prevented tumor growth in mouse models of HER2+ brain metastases. This innovative, intravenously delivered vectorized antibody approach is designed to activate the innate immune system, destroy metastatic breast cancer tumors, and inhibit tumor progression in the CNS. In preclinical studies, widespread transduction of brain tissue was achieved, effectuating expression of HER2-directed antibodies for extended periods, resulting in substantially reduced tumor growth in multiple orthotopic xenograft models.

Key Leadership Appointments Add to Deep R&D and Operational Experience

- In February, Voyager [announced](#) the appointment of Alfred W. Sandrock, Jr., M.D., Ph.D., to its Board of Directors and a newly-formed Executive Committee of the Board consisting of Dr. Sandrock, Mr. Higgins, and Glenn Pierce, M.D., Ph.D., Voyager's interim chief scientific officer. Dr. Sandrock is working with Voyager leadership to help shape future strategies leveraging the Company's TRACER™ platform and therapeutic programs, and to expand its external collaborations with scientific leaders and industry partners. Dr. Sandrock joined Voyager following a prolific career in biopharmaceutical drug development.
- The Company also recently announced the promotion of Robin Swartz to chief operating officer. Ms. Swartz joined Voyager in 2021 following a 25-year career with Genzyme and Sanofi Genzyme where she held a number of leadership positions, including Senior Vice President of U.S. and Global Business Operations, Senior Vice President U.S. Rare Disease Patient and Product Services, and Senior Director Finance.

Fourth Quarter and Full Year 2021 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$28.1 million for the fourth quarter of 2021, compared to \$6.5 million for the same period in 2020. The increase in collaboration revenue was largely due to a change in estimate related to our collaboration with Neurocrine Biosciences. For the year ended December 31, 2021, collaboration revenue was \$37.4 million, compared to \$171.1 million for the same period of 2020. The decrease in collaboration revenue is largely due to \$105.2 million of revenue related to the AbbVie collaborations that was recognized upon the termination of the collaborations during 2020.
- **Net Loss:** Net income was \$5.7 million for the fourth quarter of 2021, compared to a net loss of \$15.9 million for the same period of 2020. Net income was higher driven by the recognition of revenue related to our collaboration with Neurocrine Biosciences. Net loss was \$71.2 million for the year ended December 31, 2021, compared to net income of \$36.7 million for the same period of 2020. Voyager had net income for 2020 driven primarily by revenue related to the AbbVie collaborations which were terminated during 2020.
- **R&D Expenses:** Research and development expenses were \$14.0 million for the fourth quarter of 2021, compared to \$22.0 million for the same period in 2020. For the year ended December 31, 2021, R&D expenses were \$73.8 million, compared to \$108.8 million for the same period of 2020. The decrease in R&D expenses was related to employee-related expenses driven by a reduction in headcount as well as lower external costs primarily for clinical development and manufacturing.
- **G&A Expenses:** General and administrative expenses were \$8.4 million for the fourth quarter of 2021, compared to \$8.3 million for the same period in 2020. For the year ended December 31, 2021, G&A expenses were \$37.2 million, compared to \$35.0 million for the same period of 2020. The increase in G&A expenses was primarily related to employee-related severance expenses in connection with a reduction-in-force commenced in the third quarter of 2021 and facility costs.

- **Cash Position:** Cash, cash equivalents and marketable securities as of December 31, 2021 were \$132.5 million. This cash position does not include the \$54 million upfront payment Voyager is entitled to receive from Novartis as part of the Novartis license option agreement announced on March 8, 2022.

Financial Guidance

- Voyager expects that its cash, cash equivalents, and marketable securities inclusive of upfront payments anticipated to be received under the Novartis license option agreement will be sufficient to meet Voyager's planned operating expenses and capital expenditure requirements into 2024.

Participation in Upcoming Investor Event

- Cowen 42nd Annual Health Care Conference, corporate panel [discussion](#), Mar. 9, 2022, 12:50 p.m. ET.

Conference Call

The Voyager Therapeutics leadership team will host a conference call and webcast today at 8:30 a.m. ET to provide and discuss the fourth quarter and full year 2021 financial and operating results and the Novartis license option agreement. To access the call, please dial (877) 851-3834 (domestic) or (631) 291-4595 (international) and ask for the Voyager Therapeutics earnings call. A live webcast of the call will also be available on the Investors section of the Voyager website at ir.voyagertherapeutics.com, and a replay will be available at the same link approximately two hours after its completion. The replay will be available for at least 30 days following the conclusion of the call.

About the TRACER™ AAV Capsid Discovery Platform

Voyager's TRACER™ capsid discovery platform is a broadly applicable, RNA-based functional screening platform that allows for rapid in vivo evolution of AAV9- and AAV5-derived capsids with enhanced tropisms and cell- and tissue-specific transduction properties in multiple species, including non-human primates (NHPs). Initial data from the first of many libraries screened in NHPs demonstrated the proprietary capsid variants effectively penetrated the blood-brain barrier and achieved widespread biodistribution and transduction of multiple regions of the brain. Separate results have demonstrated the ability of certain capsids to transduce cardiac muscle and to de-target the dorsal root ganglia. Voyager is proceeding with additional capsid campaigns derived from AAV9, AAV5, and other capsid serotypes to identify novel AAV vectors optimized for specific therapeutic applications.

About Voyager Therapeutics

Voyager Therapeutics (Nasdaq: VYGR) is leading the next generation of AAV gene therapy to unlock the potential of the modality to treat devastating diseases. Proprietary capsids born from the Company's TRACER discovery platform are powering a rich early-stage pipeline of new and second-generation programs and may elevate the field to overcome the narrow therapeutic window associated with conventional gene therapy vectors across neurologic disorders and other therapeutic areas. voyagertherapeutics.com [LinkedIn](#) [Twitter](#)

Forward-Looking Statements

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "may," "might," "will," "would," "should," "expect," "plan," "anticipate," "believe," "estimate," "undoubtedly," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding Voyager's ability to continue to identify and develop proprietary capsids from its TRACER capsid discovery platform with increased transgene expression, increased blood-brain barrier penetration and increased biodistribution compared to conventional AAV9 and AAV5 capsids; Voyager's ability to utilize its novel proprietary capsids in its own product development programs; Voyager's ability to attract parties to license its novel proprietary capsids or to participate with Voyager in research and development collaborations utilizing its novel proprietary capsid; Voyager's ability to advance its AAV-based gene therapy programs; Voyager's ability to perform its obligations under its respective license option agreements with Novartis and Pfizer; Voyager's entitlement to receive upfront, option exercise, milestone and royalty-based fees from Novartis and Pfizer under the respective license option agreements; Voyager's ability to maintain its current partnerships and collaborations and to enter into new partnerships or collaborations; Voyager's ability to continue to advance and to receive regulatory approvals for product candidates under development in Voyager's pipeline; Voyager's anticipated financial results, including the receipt by Voyager of revenues or reimbursement payments from collaboration partners; and Voyager's ability to generate sufficient cash resources to enable it to continue to identify and develop proprietary capsids from its TRACER capsid discovery platform are forward looking.

All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes such forward-looking statements to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Voyager expected. Such risks and uncertainties include, among others, the severity and length of the COVID-19 health crisis, the imposition of governmental controls and guidance addressing the COVID health crisis, and the financial and human resources available to Voyager to manage the COVID-19 health crisis; the continued development of various technology platforms, including Voyager's TRACER platform; the development by third parties of capsid identification platforms that may be competitive to Voyager's TRACER capsid discovery platform; Voyager's scientific approach and general development progress; Voyager's ability to attract and retain talented contractors and employees to continue the development of the TRACER capsid discovery platform and the identification of proprietary capsids; Voyager's ability to create and protect intellectual property rights associated with the TRACER capsid discovery platform and the capsids identified by the platform; the response of the FDA and other regulators to Voyager's regulatory submissions and communications; the ability to attract and retain talented contractors and employees, including key scientists and business leaders; the ability to create and protect intellectual property; Voyager's ability to perform its obligations under its license option agreements and its counterparties' respective abilities to perform their obligations under such agreements; the sufficiency of cash resources; the possibility or the timing of the exercise of development, commercialization, license and other options under the Pfizer and Novartis license option agreements and other collaborations; the ability of Voyager to negotiate and complete licensing or collaboration agreements on terms acceptable to Voyager and third parties; and the availability or commercial potential of Voyager's product candidates.

These statements are also subject to a number of material risks and uncertainties that are described in Voyager's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, as updated by its subsequent filings with the Securities and Exchange Commission. All information in the press release is as of the date of this press release, and any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise this information or any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Selected Financial Information
(\$-amounts in thousands, except per share data)
(Unaudited)

Statement of Operations Items:	Three Months Ended December 31,		Year Ended December 31,	
	2021	2020	2021	2020
Collaboration revenue	\$ 28,073	\$ 6,537	\$ 37,415	\$ 171,128
Operating expenses:				
Research and development	14,020	21,996	73,787	108,753
General and administrative	8,351	8,270	37,246	34,991
Total operating expenses	22,371	30,266	111,033	143,744
Operating income (loss)	5,702	(23,729)	(73,618)	27,384
Total other income	6	7,802	2,421	9,357
Net income (loss)	\$ 5,708	\$ (15,927)	\$ (71,197)	\$ 36,741
Net income (loss) per share, basic	\$ 0.15	\$ (0.43)	\$ (1.89)	\$ 0.99
Net income (loss) per share, diluted	0.15	(0.43)	(1.89)	0.98
Weighted-average common shares outstanding, basic	37,804,372	37,290,259	37,668,947	37,132,447
Weighted-average common shares outstanding, diluted	37,804,372	37,290,259	37,668,947	37,348,514

Selected Balance Sheet Items	December 31,	
	2021	2020
Cash, cash equivalents, and marketable securities	\$ 132,539	\$ 174,782
Total assets	\$ 193,855	\$ 261,584
Accounts payable and accrued expenses	\$ 11,524	\$ 14,839
Deferred revenue	\$ 42,096	\$ 43,817
Total stockholders' equity	\$ 95,055	\$ 154,320

Contacts

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Source: Voyager Therapeutics, Inc.