



Voyager Therapeutics Reports First Quarter 2022 Financial and Operating Results

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Company to present new data at the American Society for Gene and Cell Therapy (ASGCT) Annual Meeting highlighting cross-species translatability and CNS tropisms of novel TRACER™ AAV9- and AAV5-derived capsids

Preclinical data to be presented at the ASGCT Annual Meeting to demonstrate reduction of tumor burden with vectorized HER2 antibodies; updated data from GBA1, vTau, SOD1 ALS programs also to be presented

Executed license option agreement with Novartis for target-specific access to next-generation TRACER™ AAV capsids

Appointed Alfred W. Sandrock, Jr., M.D., Ph.D. as Chief Executive Officer and Board Member

Cash runway into 2024

CAMBRIDGE, Mass., May 04, 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 first quarter 2022 financial and operating results.

"I'm thrilled to be leading Voyager as we advance our pioneering journey to help make AAV gene therapy a reality for patients by enabling safer, more targeted therapies," said Alfred W. Sandrock, Jr., M.D., Ph.D., chief executive officer of Voyager. "Our license option agreement with Novartis further validates the TRACER capsid discovery platform, expands the range of programs that may utilize our novel AAV capsids, and demonstrates TRACER's potential for offering future business development opportunities. We look forward to providing updates at the upcoming ASGCT Annual Meeting, including exciting new data on our AAV9- and AAV5-derived capsids demonstrating cross species translatability and unique CNS tropisms, as well as preclinical data highlighting advances in several of our pipeline programs."

Data from Evolving TRACER AAV9- and AAV5-Derived Capsid Portfolio to be Presented at ASGCT Annual Meeting

- Voyager continues to perform screening campaigns with its TRACER (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) 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. These capsids offer the potential to broaden the therapeutic window for certain gene therapies in a wide 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.
- The Company is scheduled to provide an oral presentation at the upcoming ASGCT Annual Meeting detailing a family of cross-species AAV9 derivatives with enhanced brain tropism following systemic administration. The novel, TRACER-generated AAV9-derived capsid family achieved 50-fold better transduction in mice and 60-fold better transduction in macaques compared to conventional AAV9 with a unique tropism for glial cells.
- The Company is scheduled to present data at the ASGCT Annual Meeting on a novel, TRACER-generated AAV5-derived capsid that demonstrated 20-fold higher brain transduction and 5-fold higher spinal cord transduction compared to conventional AAV9 when administered intravenously to NHPs.

Preclinical Data from Multiple Pipeline Programs to be Presented at ASGCT Annual Meeting

- Voyager continues to advance its preclinical programs and believes its TRACER capsids may enable new, best-in-class gene therapy programs with systemic intravenous (IV) delivery with lower risk of dose-limiting toxicities. Voyager's 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).
- At the ASGCT Annual Meeting, the Company is scheduled to present preclinical data on the following pipeline programs:
 - *Vectorized HER2 antibody (oral presentation)* – A CNS-targeted, TRACER-generated AAV-derived capsid was used to deliver a vectorized HER2 antibody with broad brain expression, activating the innate immune response with increased tumor-proximal natural killer cells and dendritic cells, proliferating microglia, and significantly reducing tumor burden with prolonged survival in multiple mouse models.
 - *AAV-GBA1 gene replacement therapy* – A single IV dose of a novel, blood brain barrier-penetrant GBA1 gene replacement therapy achieved widespread CNS distribution in mice and showed dose-dependent increases in GCase activity.
 - *Vectorized anti-tau antibody* – Systemic dosing of a vectorized novel anti-tau antibody targeting the C-terminal domain of tau resulted in reduced tau pathology in mouse models, and may represent a new single-dose therapeutic strategy for treating various tauopathies.
 - *SOD1 knockdown gene therapy* – IV delivery of a blood brain barrier-penetrant AAV capsid containing an RNAi SOD1-targeting gene therapy improved motor performance and survival in a SOD1-ALS mouse model.

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

- In March, Voyager [announced](#) a license option agreement with Novartis AG (NYSE: NVS) effective March 4, 2022 through which Novartis may exercise options to license novel AAV capsids generated from Voyager's TRACER capsid discovery platform for potential use with up to three undisclosed CNS targets and options to access capsids for two additional targets to be agreed on in the future, subject to availability and the payment of an additional fee. These targets are distinct from those being explored in Voyager's internal pipeline and other licensing agreements.
- Voyager received a \$54 million up front access fee and is entitled to receive up to \$37.5 million in exercise fees in the aggregate for options on three initial CNS targets, exercisable by Novartis within 12 months of the effective date of this agreement, as well as up to \$61 million in the aggregate in selection and exercise fees for two additional targets, to be agreed upon in the future. 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.
- This agreement represents the second major transaction leveraging Voyager's TRACER capsid discovery platform, following the license option agreement [announced](#) with Pfizer Inc. in October 2021.

Alfred W. Sandrock, Jr., M.D., Ph.D., Appointed Chief Executive Officer

- In March, Voyager [announced](#) the appointment of Alfred W. Sandrock, Jr., M.D., Ph.D., as chief executive officer (CEO). Dr. Sandrock succeeded Michael Higgins, who served as interim CEO from June 2021 and continues to serve as Chairman of the Voyager Board of Directors. Dr. Sandrock, who joined Voyager as a Board director in February, arrived at the company following a prolific career in biopharmaceutical drug development.
- In February, the Company 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.

First Quarter 2022 Financial Results

- **Collaboration Revenues:** Voyager had collaboration revenue of \$0.7 million for the first quarter of 2022, compared to \$6.5 million for the same period in 2021. The decrease in collaboration revenue was largely due to the termination of the Parkinson Disease program as part of the Neurocrine Biosciences collaboration agreement.
- **Net Loss:** Net loss was \$21.3 million for the first quarter of 2022, compared to a net loss of \$21.6 million for the same period of 2021. The comparable net loss was a result of lower expenses for the first quarter of 2022 compared to the first quarter of 2021, offset by lower revenues for the same period.
- **R&D Expenses:** Research and development expenses were \$14.3 million for the first quarter of 2022, compared to \$22.3 million for the same period in 2021. The decrease in R&D expenses was primarily a result of lower clinical and manufacturing spend related to the Huntington's disease program.
- **G&A Expenses:** General and administrative expenses were \$7.7 million for the first quarter of 2022, compared to \$9.7 million for the same period in 2021. The decrease in G&A expenses was primarily a result of lower facilities and related costs, as well as lower legal and intellectual property related expenses.
- **Cash Position:** Cash, cash equivalents and marketable debt securities as of March 31, 2022 were \$166.8 million, compared to \$132.5 million as of December 31, 2021.

Financial Guidance

- Voyager expects that its cash, cash equivalents, and marketable debt securities will be sufficient to meet Voyager's planned operating expenses and capital expenditure requirements into 2024.

Participation in Upcoming Scientific Conferences

- ASGCT Annual Meeting, May 16-19, 2022
- Alzheimer's Association International Conference, July 31 – August 4, 2022

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 AAV 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](#)

Voyager Therapeutics® is a registered trademark, and TRACER™ is a trademark, of Voyager Therapeutics, Inc.

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 the participation of scientists associated with Voyager making presentations at 2022 ASGCT annual meeting, the presentations of data at the 2022 ASGCT annual meeting, and participation in future scientific conferences, 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 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 final acceptance by the organizers of the 2022 ASGCT Annual Meeting of submissions presenting Voyager research; the ability of Voyager scientists to effectively deliver their presentations at 2022 ASGCT Annual Meeting; Voyager’s ability to manage the financial and human resources challenges arising from 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)

	Three Months Ended	
	March 31,	
	2022	2021
Statement of Operations Items:		
Collaboration revenue	\$ 658	\$ 6,501
Operating expenses:		
Research and development	14,349	22,346
General and administrative	7,659	9,744
Total operating expenses	22,008	32,090
Operating loss	(21,350)	(25,589)
Total other income	31	3,940
Net loss	\$ (21,319)	\$ (21,649)
Net loss per share, basic and diluted	\$ (0.56)	\$ (0.58)
Weighted-average common shares outstanding, basic and diluted	38,049,430	37,501,065

	March 31,	December 31,
	2022	2021
Selected Balance Sheet Items		
Cash, cash equivalents, and marketable debt securities	\$ 166,833	\$ 132,539
Total assets	\$ 224,182	\$ 193,855
Accounts payable and accrued expenses	\$ 8,935	\$ 11,524
Deferred revenue	\$ 95,443	\$ 42,096
Total stockholders’ equity	\$ 75,931	\$ 95,055

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Source: Voyager Therapeutics, Inc.