Voyager Therapeutics Reports Third Quarter 2021 Financial and Operating Results

November 2, 2021

License option agreement with Pfizer provides Voyager $30M up front and up to $600M in potential exercise fees and milestone payments plus royalties in exchange for access to TRACER™ novel capsids for use with two Pfizer transgenes in neurologic and cardiovascular disease

TRACER platform identifies AAV9 and AAV5 capsid variants with broad CNS tropism and neuronal and glial cell targeting in non-human primates

Preclinical data demonstrate GBA1 gene therapy achieved widespread CNS distribution and sustained correction of GCase activity in multiple brain regions in mice

CAMBRIDGE, Mass., Nov. 02, 2021 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a gene therapy company developing life-changing treatments and next-generation adeno-associated virus (AAV) technologies, today reported third quarter 2021 financial and operating results.

"We've taken notable steps in recent months to execute on our new strategy, including our recent capsid licensing agreement with Pfizer, continued progress with our RNA-driven AAV TRACER screening platform, and initial data from our GBA1 gene replacement program," said Michael Higgins, interim CEO of Voyager. "The licensing transaction with Pfizer showcases TRACER's ability to produce not only enhanced blood-brain barrier penetrant capsids, but also novel capsids with enhanced tropism for cardiac muscle, offering promise to unlock the fullest potential of gene therapies for a wide array of diseases. We've retained all rights to our capsid library outside of their use with two particular Pfizer transgenes, and we believe there is significant opportunity for similar transactions leveraging the entirety of our libraries for other targets inside and outside of the CNS."

"We're pleased to also announce that in addition to our prior results with AAV9 variants, our TRACER screening campaigns have identified AAV5 capsid variants with enhanced brain and spinal cord tropism, compared to conventional AAV5 and AAV9, as well as a new class of AAV9 variants selective for glial cells, which may enable more precise targeting of CNS diseases affecting non-neuronal cells," said Glenn Pierce, M.D., Ph.D., interim CSO of Voyager. "In parallel, we're advancing an innovative gene therapy pipeline leveraging our novel TRACER capsids. Preclinical data from our GBA1 gene replacement program illustrates the potential of a single-dose, IV-administered therapy to significantly raise target protein levels in the CNS, potentially impacting Parkinson's disease, Lewy body dementia, and Gaucher disease."

Pfizer License Option Agreement Supports TRACER Platform Potential for Multiple Therapeutic Areas

- In October, Voyager and Pfizer Inc. (NYSE: PFE) entered an agreement through which Pfizer may exercise options to license novel capsids generated from Voyager's RNA-driven TRACER (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) screening technology. Under the agreement, Pfizer may evaluate novel TRACER capsids and exercise options to license up to two capsids for transgene-exclusive Pfizer gene therapy programs for neurologic and cardiovascular disease associated with those transgenes.
- The Pfizer transgenes are distinct from those planned for Voyager's internal pipeline, and Voyager retains global rights to all TRACER capsids for use with other transgenes and to all other applications of its TRACER technology.
- Voyager received a $30 million upfront payment from Pfizer 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. Voyager is also eligible to earn up to $580 million in total development, regulatory, and commercial milestones associated with licensed products incorporating the two undisclosed Pfizer transgenes together with a Voyager licensed capsid. Voyager is also eligible to receive mid- to high-single-digit tiered royalties based on net sales of Pfizer's products incorporating the licensed capsids.

TRACER Identifies AAV9 and AAV5 Capsid Variants with Broad CNS Tropism, Neuronal and Glial Cell Targeting; Screening Campaigns Advancing

- Voyager is performing further screening with its TRACER platform to identify additional proprietary AAV9- and AAV5-derived capsids targeting multiple tissue and cell types for use in gene therapies to treat a broad range of diseases.
- Numerous promising capsids have been identified from these screens, which are undergoing rigorous testing across multiple non-human primate and rodent species to evaluate their clinical translatability.
- In addition to capsids with substantially enhanced CNS tropism for neurons in non-human primates, a new array of TRACER AAV9 and AAV5 variants show robust CNS targeting in both non-human primates and rodents and demonstrate a strong tropism for glial cells, which may enable more effective targeting of certain CNS diseases.
- Capsid candidates derived from AAV5 present significant potential advantages in terms of lower prevalence of preexisting neutralizing antibodies in the general population and manufacturing. In addition, these variants demonstrate cross-species translatability among primates and rodents. Initial testing on these AAV5-derived capsids has demonstrated approximately

20-fold improvements in brain transduction compared with AAV9 in non-human primates.
- As previously disclosed, initial data from AAV9-derived TRACER capsids demonstrated robust delivery across the blood-brain barrier and widespread CNS transduction in non-human primates compared to AAV9 delivery following intravenous (IV) administration. In addition, a proprietary capsid displayed strong cardiac transduction and significant dorsal root ganglia detargeting in non-human primates, which may avoid dose-related toxicities associated with AAV9 delivery.

Positive Preclinical Data from GBA1 Program

- Voyager continues to advance its early-stage programs including multiple programs utilizing proprietary AAV capsids derived from the TRACER platform. Voyager believes these capsids may enable new, best-in-class gene therapy programs with systemic IV delivery and lower risk of dose-limiting toxicities. Pipeline programs include those for Huntington's disease, monogenic ALS (SOD1), spinal muscular atrophy, and diseases linked to GBA1 mutations, including Parkinson's disease, Lewy body dementia, and Gaucher disease.
- Voyager presented initial preclinical data from its GBA1 gene replacement program at the virtual 2021 Annual Congress of the European Society of Gene & Cell Therapy, Oct. 19-22, 2021. The results demonstrated that an IV-administered gene replacement therapy using a proprietary AAV capsid achieved widespread distribution in the CNS and peripheral tissues, and raised levels of the deficient GCase enzyme by 300% - 660% over endogenous levels in a mouse model. By comparison, increases of 30% - 50% are expected to be clinically impactful. These data support the potential of a novel therapeutic approach for diseases associated with GBA mutations, including Parkinson's disease, Lewy body dementia, and Gaucher disease.

Upcoming Events and Presentations

- Society for Neuro-Oncology 26th Annual Meeting, Nov. 18-21, 2021

Third Quarter 2021 Financial Results

- **Collaboration Revenues:** Collaboration revenue was $1.5 million for the third quarter of 2021, compared to collaboration revenue of $117.8 million for the same period of 2020. The decrease in collaboration revenue was largely due to a reduction of revenue related to research services and cost reimbursements from the collaborations with Neurocrine and AbbVie. In February 2021, Neurocrine provided notice that effective August 2, 2021 it was terminating its participation in the VY-AADC program for Parkinson's disease under the collaboration agreement between Voyager and Neurocrine, and that wind-down activities, including the termination by Neurocrine of its support for ongoing development activities for the VY-AADC program, would commence immediately. Additionally, the collaborations with AbbVie were terminated in August 2020 and resulted in recognition of $47.2 million of revenue related to the collaboration targeting tauopathies and $59.4 million of revenue related to the collaboration targeting synucleinopathies during the quarter ended September 30, 2020, as Voyager had no further service obligations at that time.
- **Net Loss:** Net loss was $25.1 million for the third quarter of 2021, compared to net income of $85.6 million for the same period of 2020.
- **R&D Expenses:** Research and development expenses were $17.9 million for the third quarter of 2021, compared to $25.0 million for the same period in 2020. The decrease in R&D expenses was primarily related to lower research and development employee-related expenses as well as manufacturing and clinical expenses for the VY-AADC program for Parkinson's disease.
- **G&A Expenses:** General and administrative expenses were $8.7 million for the third quarter of 2021, compared to $8.3 million for the same period in 2020. The increase in G&A expenses was primarily related to increased employee-related expenses in connection with a reduction in force and facility costs.
- **Cash Position:** Cash, cash equivalents, and marketable debt securities as of September 30, 2021 were $121.5 million.

Financial Guidance

- Based on Voyager's current operating plan and excluding any potential financing or business development activities in 2021, Voyager anticipates cash, cash equivalents, and marketable debt securities will be greater than $130 million at the end of 2021.

About the TRACER™ AAV Capsid Discovery Platform
Voyager's TRACER™ system 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 unique 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 technology to treat

devastating diseases. Proprietary capsids born from the Company's TRACER™ screening platform are powering a rich early-stage pipeline of new and second-generation programs and may elevate the field to overcome the limitations of 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 AAV screening platform; Voyager's ability to identify and develop proprietary capsids from its TRACER AAV screening platform with increased transgene expression, increased blood-brain barrier penetration and increased biodistribution compared to conventional AAV5 and AAV9 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 license option agreement with Pfizer; Voyager's entitlement to receive upfront, milestone and royalty based fees from Pfizer under the license option agreement; Voyager's ability to maintain its current partnerships and collaborations and to enter into new partnerships or collaborations; and Voyager's ability to generate sufficient cash resources to enable it to continue to identify and develop proprietary capsids from its TRACER AAV screening 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; Voyager's scientific approach and general development progress; the ability to attract and retain talented contractors and employees, including key scientists and business leaders; the ability to create and protect intellectual property; the sufficiency of cash resources; the possibility or the timing of the exercise of development, commercialization, license and other options under the Pfizer license option agreement 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
Statement of Operations Items:	**2021**	**2020**	**2021**	**2020**
Collaboration revenue	$ 1,482	$ 117,843	$ 9,342	$ 164,591
Operating expenses:				
Research and development	17,914	25,039	59,767	86,757
General and administrative	8,714	8,277	28,895	26,721
Total operating expenses	26,628	33,316	88,662	113,478
Operating (loss) income	(25,146)	84,527	(79,320)	51,113
Total other income	9	1,084	2,414	1,554
Net (loss) income	$ (25,137)	$ 85,611	$ (76,906)	$ 52,667
Net (loss) income per share, basic	$ (0.67)	$ 2.30	$ (2.04)	$ 1.42
Net (loss) income per share, diluted	$ (0.67)	$ 2.27	$ (2.04)	$ 1.40
Weighted-average common shares outstanding, basic	37,780,547	37,242,504	37,623,309	37,079,242

Weighted-average common shares outstanding, diluted	37,780,547	37,672,328	37,623,309	37,500,155

Selected Balance Sheet Items	September 30, 2021	December 31, 2020
Cash, cash equivalents, and marketable debt securities	$ 121,499	$ 174,782
Total assets	$ 184,774	$ 261,584
Accounts payable and accrued expenses	$ 11,916	$ 14,839
Deferred revenue	$ 39,176	$ 43,817
Total stockholders' equity	$ 87,422	$ 154,320

Source: Voyager Therapeutics, Inc.